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U.S. Senate

October 2011

NATIONAL PREPAREDNESS

Improvements Needed for Acquiring Medical Countermeasures to Threats from Terrorism and Other Sources

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Highlights of [GAO-12-121](#), a report to the Committee on Homeland Security and Governmental Affairs, U.S. Senate

Why GAO Did This Study

The United States remains vulnerable to terrorist and other threats posed by chemical, biological, radiological, and nuclear (CBRN) agents. Medical countermeasures—drugs, vaccines, and diagnostic devices—can prevent or treat the effects of exposure, but few are currently available. The Department of Health and Human Services (HHS) leads federal efforts to develop and acquire countermeasures, primarily through the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE), an interagency body. This report examines the extent to which HHS (1) based its priorities for developing and acquiring countermeasures on CBRN risk assessments; (2) addressed its own recommendations to improve acquisition and development; and (3) coordinated internally for these efforts. GAO reviewed relevant laws, agency documents, CBRN risk assessments, and reports from outside experts; interviewed HHS and industry officials; and analyzed HHS funding for CBRN countermeasures from fiscal years 2007 through 2010.

What GAO Recommends

GAO recommends that HHS update its development and acquisition plan; provide budget priorities for acquisitions; and develop a strategy to monitor implementation of its initiatives. HHS agreed with the first two recommendations. For the third one, HHS said that it had a strategy to track implementation, but in GAO's assessment, the strategy does not meet standards and practices to allow for adequate monitoring.

View [GAO-12-121](#) or key components. For more information, contact Marcia Crosse at (202) 512-7114 or crosse@gao.gov.

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NATIONAL PREPAREDNESS

Improvements Needed for Acquiring Medical Countermeasures to Threats from Terrorism and Other Sources

What GAO Found

Through PHEMCE, HHS laid out its CBRN medical countermeasure development and acquisition priorities in 2007 in a publicly available plan based primarily on two types of CBRN risk assessments—one from the Department of Homeland Security (DHS) and one from HHS—but HHS has not updated the plan as intended. The 2007 plan outlined spending for these priorities through 2013, when special federal funding for countermeasure acquisition will expire. HHS invested about \$1.9 billion in development and \$2.4 billion for acquisition of countermeasures to fulfill these priorities from fiscal year 2007 to fiscal year 2010. Since 2007, DHS and HHS have continued to assess the risks that CBRN agents pose to national security and public health, and HHS has reassessed decisions on the quantities and types of medical countermeasures needed. However, HHS has not updated its plan, as it had intended to do biennially, to indicate whether any priorities have changed. Further, HHS has not provided specific information on anticipated budget priorities for countermeasure acquisition—information desired by companies to help them decide whether to invest in product development.

HHS has begun to address most recommendations from its August 2010 review of PHEMCE and of HHS's countermeasure activities, but HHS has not developed an adequate strategy to monitor implementation. HHS's initiatives to address the recommendations are intended to improve product development and acquisition and PHEMCE's structure and management. These initiatives are led by different agencies and offices—for example, the Food and Drug Administration has begun efforts to improve its regulatory framework, while the National Institutes of Health has begun to implement a program to increase the number of potential products in the pipeline. HHS officials said they have a monitoring strategy that includes quarterly updates of a planning document and quarterly and annual reviews of progress. However, the planning document contains incomplete information and does not allow for measuring progress across all initiatives. Thus, HHS's monitoring strategy is not consistent with federal internal control standards and program management best practices. Given the initiatives' complexity and dispersed HHS leadership responsibilities, an adequate monitoring strategy would help HHS assess overall progress and provide information about whether HHS is meeting its countermeasure development and acquisition objectives.

HHS's establishment of PHEMCE in 2006 and its subsequent written agreements have facilitated intradepartmental coordination on the development and acquisition of CBRN medical countermeasures, but some coordination challenges remain. PHEMCE established an intradepartmental coordination process and documented the roles and responsibilities of its partners through written agreements. However, some industry and outside experts have reported that HHS's agencies and offices do not coordinate well to advance products through development to acquisition, which hampers industry's efforts to supply countermeasures. HHS officials are renewing the PHEMCE intradepartmental memorandum of understanding and charter for the governing body. These written agreements, once finalized, should continue to enhance and sustain intradepartmental coordination on countermeasure development and acquisition activities. In addition, effectively implementing some of the initiatives from HHS's August 2010 review may help mitigate these coordination challenges.

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Abbreviations

ASPR	Office of the Assistant Secretary for Preparedness and Response
BARDA	Biomedical Advanced Research and Development Authority
CBRN	chemical, biological, radiological, and nuclear
CDC	Centers for Disease Control and Prevention
DHS	Department of Homeland Security
DOD	Department of Defense
ESC	Enterprise Senior Council
FDA	Food and Drug Administration
HHS	Department of Health and Human Services
MOU	memorandum of understanding
MTA	material threat assessment
NBSB	National Biodefense Science Board
NIH	National Institutes of Health
PHEMCE	Public Health Emergency Medical Countermeasures Enterprise
SNS	Strategic National Stockpile
TRA	terrorism risk assessment

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G A O

Accountability * Integrity * Reliability

United States Government Accountability Office
Washington, DC 20548

October 26, 2011

The Honorable Joseph I. Lieberman
Chairman
The Honorable Susan M. Collins
Ranking Member
Committee on Homeland Security and Governmental Affairs
United States Senate

The anthrax attacks of 2001 raised concerns about the United States' vulnerability to intentional terrorist threats from chemical, biological, radiological, and nuclear (CBRN) agents, and the 2007 National Strategy for Homeland Security stated that terrorists have declared their intention to acquire and use CBRN agents as weapons to inflict catastrophic attacks against the United States.¹ More recently, the May 2010 National Security Strategy noted that the American people face no greater or more urgent danger than a terrorist attack with a nuclear weapon, and the effective dissemination of a lethal biological agent within a U.S. city would endanger the lives of hundreds of thousands of people and have unprecedented economic, societal, and political consequences.² In addition, the recent earthquake and resulting tsunami in Japan that caused a nuclear reactor to release radioactive material highlighted a population's vulnerability to unintentional CBRN exposure.

Rapid diagnosis, treatment, and prevention may minimize the public health impact of a release of a CBRN agent. Congress appropriated a total of about \$5.6 billion to be available for obligation from fiscal year 2004 through fiscal year 2013 for the Project BioShield Special Reserve Fund to acquire certain CBRN medical countermeasures, such as drugs, vaccines, and devices to diagnose, treat, prevent, or mitigate potential

¹White House, *National Strategy for Homeland Security* (Washington, D.C.: October 2007).

²White House, *National Security Strategy* (Washington, D.C.: May 2010).

effects of exposure to these agents.³ However, there are currently few available medical countermeasures. Research and development to create useable countermeasures is a lengthy, complex, and expensive process that involves public and private investment. However, the general lack of a commercial market for medical countermeasures against these agents may reduce incentives for industry—pharmaceutical and medical device manufacturers—to invest millions of dollars to develop countermeasures instead of other products that may be more lucrative.

The Department of Health and Human Services (HHS) is the federal agency primarily responsible for identifying needed medical countermeasures to prevent or mitigate potential health effects from exposure to CBRN agents and engaging with industry to develop them. In addition, because CBRN agents differ in their potential to cause widespread illness and death, the Department of Homeland Security (DHS) and HHS assess the risks and potential public health consequences of attacks with CBRN agents to identify those agents that represent the highest risk and to help guide response planning and countermeasure development. In 2006, HHS established the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE), a federal interagency body that includes various HHS agencies and offices, DHS, the Department of Defense (DOD), and others and is responsible for providing recommendations to the Secretary of HHS on medical countermeasure priorities and development and acquisition activities.⁴ However, members of Congress and several expert organizations have raised concerns about whether HHS and its agencies and offices are successfully developing and acquiring medical countermeasures to

³The Project BioShield Act of 2004 authorized the appropriation of funds for the Special Reserve Fund for the acquisition of certain CBRN medical countermeasures using the authorities granted under that law. 6 U.S.C. § 321j. Prior to enactment of this law, the Department of Homeland Security appropriations act for fiscal year 2004 had appropriated the amount of the Special Reserve Fund. Pub. L. No. 108-90, 117 Stat. 1137, 1148 (2003).

⁴From 2004 to 2006, the Executive Office of the President led interagency coordination efforts to establish CBRN medical countermeasure requirements.

respond to CBRN incidents.⁵ In December 2009, the HHS Secretary called for a comprehensive review of HHS's medical countermeasure development and acquisition activities. As a result, HHS issued an August 2010 review with several recommendations intended to improve its CBRN medical countermeasure development and acquisition efforts.⁶

You asked us to examine the extent to which HHS has developed risk-informed investment priorities and strategies for developing CBRN medical countermeasures. Our current review addresses (1) the extent to which HHS has based its priorities for and the resulting investments in medical countermeasure development and acquisition on CBRN risk assessments, (2) the extent to which HHS has addressed its own recommendations to improve its CBRN medical countermeasure development and acquisition activities, and (3) the extent to which HHS's agencies and offices have coordinated with each other to develop and acquire CBRN medical countermeasures.

To determine the extent to which HHS has based its medical countermeasure development and acquisition priorities and investments on CBRN risk assessments, we analyzed relevant laws and presidential directives to determine requirements for HHS to use CBRN risk

⁵See House Committee on Homeland Security, Subcommittee on Emergency Preparedness, Response, and Communication, *Taking Measure of Countermeasures (Part 1): A Review of Government and Industry Efforts to Protect the Homeland through Accelerated Research, Development, and Acquisition of Chemical, Biological, Radiological and Nuclear Medical Countermeasures*, 112th Cong., 1st sess., 2011; Senate Committee on Appropriations, Subcommittee on Labor, Health and Human Services, and Education, and Related Agencies, *Defending Against Public Health Threats*, 111th Cong., 2nd sess., 2010; Senate Committee on Homeland Security and Governmental Affairs, *Six Years after Anthrax: Are We Better Prepared to Respond to Bioterrorism?* 110th Cong., 1st sess., 2007; and House Committee on Homeland Security, Subcommittee on Emerging Threats, Cybersecurity, and Science and Technology, *Can BioShield Effectively Procure Medical Countermeasures That Safeguard the Nation?* 110th Cong., 1st sess., 2007.

⁶See Department of Health and Human Services, Assistant Secretary for Preparedness and Response, *The Public Health Emergency Medical Countermeasures Enterprise Review: Transforming the Enterprise to Meet Long-Range National Needs* (Washington, D.C.: August 2010).

assessments for medical countermeasure decision making.⁷ We analyzed DHS and HHS CBRN risk assessments and other HHS documents on stated countermeasure development and acquisition priorities developed since 2004 to determine whether the stated priorities align with the highest-risk agents identified in the risk assessments. We also interviewed officials from HHS agencies and offices—including the Office of the Assistant Secretary for Preparedness and Response (ASPR), the Biomedical Advanced Research and Development Authority (BARDA), the National Institutes of Health (NIH), the Centers for Disease Control and Prevention (CDC), and the Food and Drug Administration (FDA)—to obtain information on how HHS officials determined priorities for countermeasures, and whether and how HHS officials used CBRN risk assessments to do so. In addition, to determine how much funding HHS has invested in CBRN medical countermeasure development and acquisition from fiscal year 2007 through fiscal year 2010, we analyzed agency data and reports on BARDA, CDC, and NIH investments, compared these investments with HHS’s stated countermeasure priorities and with its acquisitions, and interviewed HHS officials.⁸ We chose this period because it coincided with the beginning of the period covered by HHS’s plan for developing and acquiring CBRN medical countermeasures. The overall estimates of annual NIH investments for CBRN medical countermeasure research and development that we are reporting are based on information provided by NIH. NIH investments may have multiple components, only some of which may be related to CBRN medical countermeasure development. NIH provided us with estimates of the amounts related to specific types of CBRN research, based on percentages of research awards. We cross-checked publicly available information on funding for medical countermeasure development and acquisition with the funding information provided by HHS and interviewed agency officials about how they ensure the accuracy of the funding information provided, but did not independently verify the funding information provided by HHS. Through these steps, we

⁷For the purposes of this report, we consider CBRN risk assessments to include DHS’s terrorism risk assessments (TRA) and material threat assessments (MTA) and HHS’s public health and medical consequence modeling reports. TRAs assess the risks posed by CBRN agents based on variable threats, vulnerabilities, and consequences. MTAs assess the threat posed by given CBRN agents and the potential number of human exposures in plausible high-consequence scenarios. Modeling reports assess the public health and medical consequences of attacks with CBRN agents for given scenarios.

⁸For the purposes of this report, we used the term “invested” to mean obligated.

determined that the data we received from HHS were sufficiently reliable for our purposes.

To determine the extent to which HHS has addressed its own recommendations to improve its CBRN medical countermeasure development and acquisition activities, we analyzed HHS's August 2010 review of these activities to identify recommendations the department made that were specific to CBRN countermeasure research, development, and acquisition.⁹ We reviewed other HHS documentation, such as the department's fiscal year 2012 budget requests, and interviewed HHS officials to determine how HHS planned to implement initiatives to address the recommendations, including which agencies and offices are responsible for specific initiatives, time frames for and status of implementation, and any barriers to implementation. In order to assess HHS's overall plans for implementing initiatives to address the recommendations, we compared HHS's plans to federal standards for internal control and the Project Management Institute's *The Standard for Program Management* for program management best practices.¹⁰

To determine the extent to which HHS's agencies and offices have coordinated to develop and acquire CBRN medical countermeasures, we reviewed HHS policies and procedures, including memorandums of understanding, charters, and other documents, and expert assessments of HHS's CBRN medical countermeasure development and acquisition activities and interviewed HHS officials. We also conducted interviews with six industry officials from five companies who were knowledgeable about HHS's medical countermeasure activities. We selected these officials based on their experience working with HHS on advanced countermeasure development or acquisition, from companies that varied in size and products offered. Through these steps we identified guidance

⁹See HHS, *The Public Health Emergency Medical Countermeasures Enterprise Review*. We did not report on recommendations from this report that related solely to pandemic influenza. We have previously examined how HHS intends to address some of its recommendations in the review related to pandemic influenza vaccine. See GAO, *Influenza Pandemic: Lessons Learned from the H1N1 Pandemic Should be Incorporated into Future Planning*, [GAO-11-632](#) (Washington, D.C.: June 27, 2011); and *Influenza Vaccine: Federal Investments in Alternative Technologies and Challenges to Development and Licensure*, [GAO-11-435](#) (Washington, D.C.: June 27, 2011).

¹⁰See GAO, *Standards for Internal Control in the Federal Government*, [GAO/AIMD-00-21.3.1](#) (Washington, D.C.: November 1999); and Project Management Institute, *The Standard for Program Management*, 2nd ed. (Newton Square, Pa.: 2008).

for intradepartmental coordination, used them to assess the extent to which HHS agencies coordinate to develop and acquire CBRN medical countermeasures and to understand any barriers to this coordination, and compared HHS's coordination practices with our best practices for enhancing and sustaining agency collaboration.¹¹

We conducted this performance audit from February 2011 through October 2011 in accordance with generally accepted government auditing standards. Those standards require that we plan and perform the audit to obtain sufficient, appropriate evidence to provide a reasonable basis for our findings and conclusions based on our audit objectives. We believe that the evidence obtained provides a reasonable basis for our findings and conclusions based on our audit objectives.

Background

HHS is responsible for identifying needed medical countermeasures to prevent or mitigate the potential health effects from exposure to CBRN agents and researching, developing, and acquiring these countermeasures. The Project BioShield Act of 2004 authorized the appropriation of a total of about \$5.6 billion from fiscal years 2004 through 2013.¹² The act facilitated the creation of a government market by authorizing the government to commit to make the Special Reserve Fund available to purchase certain medical countermeasures,¹³ including those

¹¹These practices require that components of an organization's management provide reasonable assurance that certain objectives, including effectiveness and efficiency of operations, are being achieved. See GAO, *Results-Oriented Government: Practices That Can Help Enhance and Sustain Collaboration among Federal Agencies*, [GAO-06-15](#) (Washington, D.C.: October 21, 2005), and [GAO/AIMD-00-21.3.1](#).

¹²6 U.S.C. § 321j. Prior to enactment of this law, the Department of Homeland Security appropriations act for fiscal year 2004 had appropriated the amount of the Special Reserve Fund. Pub. L. No. 108-90, 117 Stat. 1137, 1148 (2003). In 2009, the Consolidated Appropriations Act for fiscal year 2010 transferred the remaining balance of the fund to HHS. Pub. L. No. 111-117, 123 Stat. 3034, 3255 (2009).

¹³42 U.S.C. §§ 247d-6b(c)(1), (4). The Project BioShield Act also authorizes the federal government to use specific contracting authorities to procure certain medical countermeasures for CBRN agents and requires HHS to report on its use of these contracting authorities and procurements using the Special Reserve Fund. 42 U.S.C. §§ 247d-6b(c)(7), 247d-6c.

countermeasures that may not yet be FDA-approved or licensed.¹⁴ The act also allowed the HHS Secretary to authorize, under specified conditions, the temporary emergency use of products that have not yet received FDA approval.¹⁵

In 2006, HHS established PHEMCE, a federal interagency body responsible for providing recommendations to the Secretary of HHS on medical countermeasure priorities, development and acquisition activities, and strategies for distributing and using medical countermeasures held in the U.S. Strategic National Stockpile (SNS), the national repository of medications, medical supplies, and equipment for use in a public health emergency.¹⁶ The PHEMCE working groups and senior council serve as the primary means of communication between HHS and participating federal departments on CBRN medical countermeasure issues. As required by the Pandemic and All-Hazards Preparedness Act, PHEMCE also conducts annual reviews of the contents of the SNS.¹⁷

Roles and Responsibilities of HHS's Agencies and Offices

Within HHS, several agencies and offices have specific responsibilities for CBRN medical countermeasure development and acquisition. ASPR leads PHEMCE and the medical and public health response to potential CBRN incidents, including strategic planning, medical countermeasure prioritization, and support for developing and acquiring medical countermeasures. Within ASPR, BARDA—established by the Pandemic and All-Hazards Preparedness Act of 2006—oversees advanced

¹⁴The Special Reserve Fund may be used to acquire medical countermeasures that are reasonably expected to qualify for FDA approval or licensure within 8 years. 42 U.S.C. § 247d-6b(c)(1)(B)(i)(III). Under federal law and FDA regulations, vaccines and other biologics are “licensed,” drugs are “approved,” and devices may either be “approved” or “cleared.” See 42 U.S.C. § 262, 21 U.S.C. § 355, 21 U.S.C. §§ 360e, 360(k). For this report, we use the term “approve” to refer to both approval and clearance.

¹⁵21 U.S.C. § 360bbb-3.

¹⁶In addition to these responsibilities, PHEMCE is also responsible for providing recommendations on countermeasures for pandemic influenza and other emerging infectious diseases. PHEMCE is composed primarily of officials from HHS's ASPR, CDC, FDA, and NIH, which have specific responsibilities for countermeasure development and acquisition. PHEMCE also includes officials from other federal departments and offices, such as DHS, DOD, the Department of Veterans Affairs, the Department of Agriculture, and the Executive Office of the President.

¹⁷42 U.S.C. § 247d-6b(a)(1).

development and acquisition of some CBRN medical countermeasures into the SNS.¹⁸ The National Biodefense Science Board (NBSB), established by the Pandemic and All-Hazards Preparedness Act, is an advisory committee composed of 13 voting members with expertise in science, medicine, and public health that provides the HHS Secretary with expert advice and guidance on scientific and technical matters related to current and future CBRN agents, including those that occur naturally.¹⁹ NIH conducts and funds basic and applied research to develop new or enhanced medical countermeasures and related medical tools to protect the nation against threats posed by CBRN agents. CDC maintains the SNS and supports state and local public health departments' efforts to detect and respond to public health emergencies, including providing guidance and recommendations for the mass distribution and use of medical countermeasures. FDA assesses the safety and effectiveness of CBRN medical countermeasures and regulates their development, approval or licensure, and postmarket surveillance. FDA also provides technical support for the creation of tools to support medical countermeasure development and may authorize the emergency use of medical products that have not yet been approved or licensed or were approved or licensed only for other uses.

DHS and HHS CBRN Risk Assessments

As part of its preparedness role to plan and coordinate the federal interagency response to catastrophic CBRN incidents, DHS develops two types of CBRN risk assessments—terrorism risk assessments (TRA) and material threat assessments (MTA)—with some input from HHS. The requirements for these departments to develop such risk assessments are in provisions in the Project BioShield Act of 2004 and Homeland Security Presidential Directives 10 (Biodefense for the 21st Century), 18 (Medical Countermeasures Against Weapons of Mass Destruction), and 22 (Domestic Chemical Defense). TRAs assess the relative risks posed by multiple CBRN agents based on variable threats, vulnerabilities, and

¹⁸42 U.S.C. § 247d-7e. The act also gave BARDA the authority to make advance and milestone-based payments to vendors prior to product delivery to the SNS. 42 U.S.C. § 247d-7e(c)(5)(C), (D).

¹⁹42 U.S.C. § 247d-7f. Additionally, the National Biodefense Science Board, includes nonvoting members, as deemed appropriate by the Secretary.

consequences, and DHS updates TRAs regularly.²⁰ MTAs assess the threat posed by given CBRN agents or classes of agents and the potential number of human exposures in plausible, high-consequence scenarios. DHS uses the MTAs to determine which CBRN agents pose a material threat sufficient to affect national security.²¹

The Project BioShield Act of 2004 calls for HHS to assess the public health consequences of exposure to those CBRN agents that DHS determines are material threats to the nation and to determine for which of these agents medical countermeasures are necessary to protect the public's health.²² HHS's public health consequence modeling reports use the exposure information from DHS's MTAs to calculate the number of individuals who may become ill, be hospitalized, or die based on the MTA scenario. These modeling reports represent an interim step in determining needed countermeasures. HHS uses the modeling reports as part of an assessment process to establish requirements for medical countermeasures that need to be developed and acquired to respond to a CBRN incident.

Medical Countermeasure Research, Development, Acquisition, and Support

HHS's and PHEMCE's medical countermeasure acquisition strategy is based on a multistep process. This process includes assessing the threat and public health consequences of CBRN agents, determining the type and quantity of needed medical countermeasures, evaluating the public health response capability, and developing and acquiring countermeasures against high-risk CBRN agents for the SNS. Because desired CBRN medical countermeasures may not be developed to a point where they are available for acquisition, HHS oversees and supports research and development of these countermeasures. (See fig. 1.) NIH

²⁰According to the *DHS Risk Lexicon*, threats are entities, actions, or occurrences, whether natural or man-made, that have or indicate the potential to harm life, information, operations and/or property; vulnerabilities are physical features or operational attributes that render an entity, asset, system, network, or geographic area susceptible or exposed to hazards; and consequences are potential or actual effects of an event, incident, or occurrence. Department of Homeland Security (DHS), *DHS Risk Lexicon: 2010 Edition* (Washington, D.C.: September 2010).

²¹See GAO, *Public Health Preparedness: Developing and Acquiring Medical Countermeasures Against Chemical, Biological, Radiological, and Nuclear Agents*. [GAO-11-567T](#) (Washington, D.C.: April 13, 2011).

²²42 U.S.C. § 247d-6b(c)(2)(B).

and BARDA oversee and support CBRN medical countermeasure research and development, which is conducted in several stages: (1) basic research, (2) applied research, (3) early development, and (4) advanced development.²³ NIH typically provides federal funding for basic and applied research and early development.²⁴ BARDA typically funds advanced development of medical countermeasures.²⁵ If a countermeasure is not FDA-approved or licensed, its acquisition into the SNS is typically funded by the Project BioShield Special Reserve Fund.²⁶ If a countermeasure is FDA-approved or licensed, CDC purchases the countermeasure for the SNS.

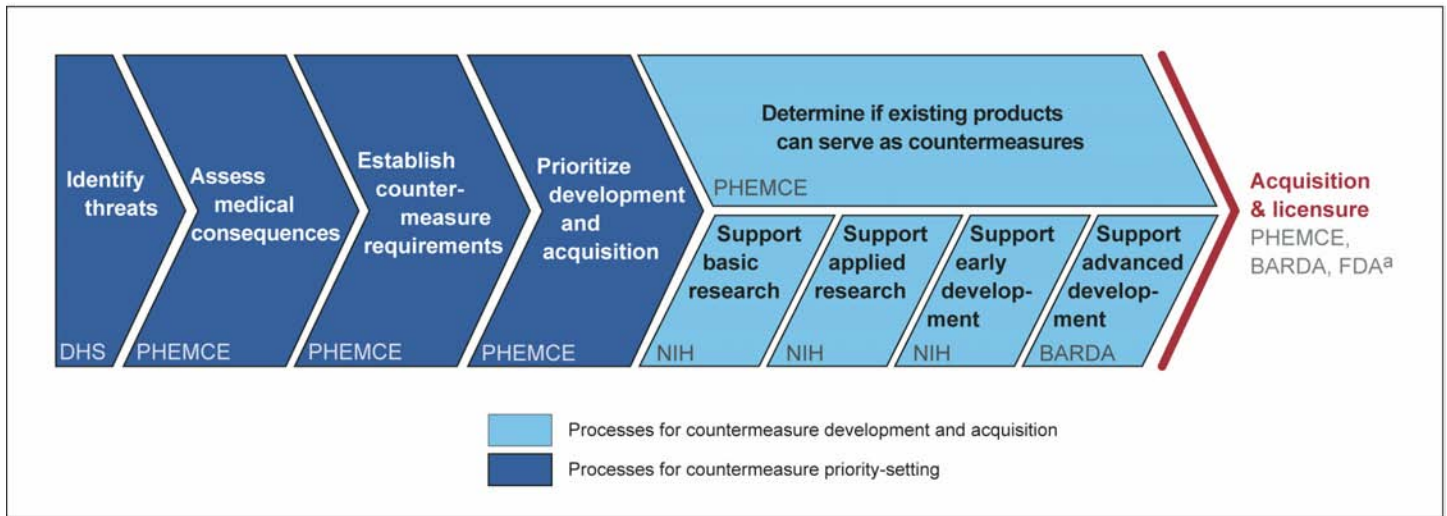
²³In addition to approving or licensing medical countermeasures, FDA works with researchers throughout the development stages to review safety and effectiveness test results and provide technical assistance to help ensure that research meets FDA's regulatory requirements.

²⁴Early, or basic, research seeks to better understand CBRN agents and the response of the host organism to the agents through the study of the cellular and molecular biology of agents and hosts, their physiologic processes, and their genome sequences and structures. Applied, or translational, research builds on basic research by validating and testing concepts in practical settings to identify potential products. Successful concepts move from the applied research stage into the early development stage, in order to demonstrate basic safety, reproducibility, and ability to be used in humans.

²⁵In the advanced development stage, potential medical countermeasures are further evaluated to demonstrate safety and effectiveness for preventing, diagnosing, or treating disease. Successful products are then available for development and acquisition. In addition, BARDA determines that manufacturing, scale-up production, and licensing of countermeasures can be achieved in a timely and reliable manner.

²⁶The Project BioShield Act of 2004 provides that the Special Reserve Fund may be used to acquire countermeasures for which the HHS Secretary determines the scientific research supports a reasonable conclusion that the product will qualify for FDA approval or licensing within 8 years. 42 U.S.C. § 247d-6b(c)(1)(B)(i)(III)(bb).

Figure 1: Processes for Medical Countermeasure Development and Acquisition



Source: GAO analysis of HHS information.

^aIn addition to approving or licensing medical countermeasures, FDA works with researchers throughout the development stages to review safety and effectiveness test results and provide technical assistance to help ensure that research meets FDA's regulatory requirements.

HHS Uses CBRN Risk Assessments in Determining Investment Priorities for Developing and Acquiring Countermeasures but Has Not Updated These Priorities since 2007

HHS based its medical countermeasure development and acquisition priorities and investments primarily on two types of CBRN risk assessments, but the department has not updated its plan outlining these priorities since 2007. Through PHEMCE, HHS laid out its countermeasure priorities in a publicly available plan in 2007. The priorities included countermeasures for every agent that DHS considered a material threat to national security. From fiscal year 2007 through fiscal year 2010, HHS invested about \$4.3 billion in countermeasure development and acquisition—\$1.9 billion in research and development and \$2.4 billion for acquisition of countermeasures to fulfill these priorities. Since 2007, DHS and HHS have continued to assess the risks that CBRN agents pose to national security and public health, and HHS has reassessed the quantities and types of medical countermeasures needed. However, HHS has not used this information to update the countermeasure priorities established in the 2007 PHEMCE plan and communicate them to pharmaceutical and medical device manufacturers and private partners.

HHS Uses Two Types of CBRN Risk Assessments as a Basis for Its Medical Countermeasure Priorities and Has Invested \$4.3 Billion in Their Development and Acquisition

HHS based its priorities for CBRN medical countermeasure development and acquisition investments primarily on two types of risk assessments, DHS's MTAs and HHS's public health consequence modeling reports. DHS used the MTAs to determine which agents pose material threats to national security. Using information from DHS's MTAs, HHS assessed the public health consequences of attacks with those agents by modeling the health effects from exposure to the agents according to the attack scenario in the MTAs. Based on the provisions of the Project BioShield Act of 2004, HHS determined whether medical countermeasures were needed for the agents DHS determined to pose material threats. HHS officials told us they also consulted with experts and reviewed public health literature to determine needed quantities, types, and desired characteristics of countermeasures. HHS has generally not used DHS's TRAs to help determine which countermeasures to develop and acquire. HHS officials told us that the TRAs do not provide enough specific information, such as estimates of the number of people potentially exposed to particular agents, to be useful in assessing public health consequences and determining requirements for the needed types and quantities of medical countermeasures.²⁷

In 2007, HHS released the PHEMCE implementation plan, a public document containing its CBRN medical countermeasure development and acquisition priorities. In the 2007 plan, HHS assigned priority to developing and acquiring particular countermeasures for all CBRN agents that DHS deemed material threats up to that time and for an additional chemical agent, based on DHS's MTAs and HHS's public health consequence modeling reports. (See app. I, table 2, for HHS's countermeasure priorities and time frames from the 2007 plan.) For example, HHS priorities include the need to develop and acquire diagnostic tests for several agents, such as anthrax, as a midterm priority.

From fiscal year 2007 through fiscal year 2010, HHS invested about \$4.3 billion in CBRN medical countermeasure research, development, and acquisition for those countermeasures that HHS determined to be

²⁷HHS officials told us that the two departments are working together to discuss how DHS may be able to make the TRAs more useful for HHS's purposes. For example, HHS officials said they were working to understand the data inputs and outputs of the integrated CBRN TRA and the biological TRA to understand how DHS determines relative risk among the agents in the TRAs and use that information to help determine needed quantities and types of medical countermeasures for specific CBRN agents.

priorities based on the MTAs and the modeling reports.²⁸ Of this \$4.3 billion, HHS invested \$1.9 billion in countermeasure research and development for those countermeasures that were not immediately available for acquisition. Of this \$1.9 billion, NIH invested \$1.2 billion in early research and early development,²⁹ and BARDA invested \$681 million in advanced development for HHS priorities. (See app. I, tables 3 and 4, for NIH and BARDA investments, respectively.) For example, from fiscal year 2007 through fiscal year 2010, NIH invested \$295 million for diagnostics and countermeasures for smallpox, \$187 million for diagnostics and countermeasures for exposure to radiological and nuclear agents, \$197 million for countermeasures for chemical agents,³⁰ and almost \$10 million for early research and development of diagnostics for glanders and melioidosis (*Burkholderia*).³¹ From fiscal years 2007 through 2010, BARDA invested \$244 million for advanced development of anthrax vaccine³² and \$146 million for advanced development of countermeasures for radiological and nuclear agents, such as antioxidants and chelating agents to remove radioactive material from the body. BARDA also invested \$53 million from fiscal years 2007 through 2009 in advanced development for smallpox countermeasures, of which \$16 million was for advanced development of

²⁸For the purposes of this report, we used the term “invested” to mean obligated.

²⁹NIH investment amounts may over- or underestimate NIH’s actual investments in CBRN countermeasure research and development. NIH officials told us that some research projects may have broader applications than for CBRN countermeasures. For example, vaccine research may be applicable to CBRN agents and to other diseases. In addition, other NIH research not included in the agency’s CBRN-specific investments may be applicable to countermeasure development, such as cancer research that may help inform research on countermeasures for exposure to radiological and nuclear agents.

³⁰NIH does not break out its investments in research and development of radiological, nuclear, and chemical agents by type of countermeasure.

³¹In addition to NIH investments in the specific countermeasures HHS identified as priorities based on the MTAs and the modeling reports, NIH has also invested in research related to other countermeasures for the 14 agents in the 2007 PHEMCE plan. For example, HHS’s only priority countermeasure identified in the plan to address botulinum toxin was for diagnostics, but NIH also invested in research on countermeasures to treat the effects of the toxin on affected individuals. NIH’s investments also include research on other potential public health threats, including foodborne diseases such as salmonella, emerging infectious diseases such as severe acute respiratory syndrome, and other agents such as ricin toxin.

³²BARDA investments in anthrax vaccine for fiscal years 2008 through 2010 included investments for two types of anthrax vaccine and portable ventilators to treat inhalation anthrax.

smallpox antivirals, which HHS subsequently acquired for the SNS in fiscal year 2011.³³

From fiscal year 2007 through fiscal year 2010, HHS invested the rest of the \$4.3 billion—approximately \$2.4 billion—to acquire available countermeasures, all of which it had identified as priorities based on the MTAs and the modeling reports.³⁴ For example, HHS invested \$1.1 billion of the Project BioShield Special Reserve Fund from fiscal years 2007 through 2010 to acquire anthrax vaccine, anthrax antitoxin, and smallpox vaccine for the SNS.³⁵ (See app. I, table 5, for HHS Project BioShield Special Reserve Fund investments.) CDC also spent \$1.3 billion from fiscal year 2007 through fiscal year 2010 to maintain the quantities of CBRN countermeasures held in the SNS. However, many of the countermeasures that HHS and PHEMCE determined to be priorities are not available. For example, no FDA-approved, rapid, point-of-care diagnostics exist for any of the biological agents deemed material threats.³⁶

³³In May 2011, BARDA invested \$433 million of the Project BioShield Special Reserve Fund to acquire 1.7 million doses of smallpox antivirals for delivery to the SNS within 5 years.

³⁴From fiscal year 2004 through fiscal year 2006, HHS spent approximately \$1 billion of the Special Reserve Fund to acquire countermeasures such as botulism antitoxin, countermeasures to combat effects of radiation exposure, and other anthrax countermeasures.

³⁵We previously reported on the status of HHS's medical countermeasure acquisitions with the Project BioShield Special Reserve Fund. See GAO, *Project BioShield Act: HHS Has Supported Development, Procurement, and Emergency Use of Medical Countermeasures to Address Health Threats*, [GAO-09-878R](#) (Washington, D.C.: July 24, 2009).

³⁶According to FDA, rapid, point-of-care diagnostics would help guide the public health response to a CBRN incident and ensure that patients receive the most appropriate treatment.

HHS Has Not Updated Its CBRN Medical Countermeasure Priorities in a Publicly Available Plan since 2007 to Make Its Current Priorities Clear to Industry

HHS has not updated the PHEMCE implementation plan since 2007 to publicly present its current priorities for CBRN medical countermeasure research, development, and acquisition. HHS stated in its 2007 PHEMCE plan that it would update the plan in 2009 and biennially thereafter to reflect any changes in threats posed by specific CBRN agents and the availability of new countermeasures. However, HHS officials told us the department's efforts to review and update the PHEMCE plan in 2009 were suspended until HHS finished an overall examination of PHEMCE in the summer of 2010. This review was completed and published in August 2010, but HHS officials told us in June 2011 that PHEMCE had not yet made key decisions on what would be included in an updated plan. In August 2011, HHS officials told us the department was in the process of establishing a steering committee, composed of PHEMCE interagency participants, which is tasked with updating the PHEMCE strategy and implementation plan. According to HHS officials, the department plans to finalize and publicly release the updated PHEMCE plan in spring 2012. In addition, HHS officials said that the next version of the plan would likely be an all-hazards plan that includes influenza and emerging infectious diseases.

The 2007 plan called for biennial review and revision of PHEMCE's countermeasure priorities to incorporate updated information from DHS's risk assessments. For example, HHS's countermeasure priorities in the anticipated 2009 plan were to be further informed by DHS's 2008 integrated CBRN TRA, which provided assessments of which CBRN agents presented the highest risk, based on DHS's individual 2006 biological and chemical TRAs. The 2007 plan also stated that future versions would incorporate more detailed assessments of needed countermeasures for enhanced, emerging, or advanced biological agents to improve preparedness against changing CBRN threats.³⁷ When HHS publicly released the PHEMCE implementation plan containing its

³⁷According to Homeland Security Presidential Directive 18, *Medical Countermeasures Against Weapons of Mass Destruction*, enhanced agents are traditional biological agents that have been modified or selected to enhance their ability to harm human populations or circumvent current countermeasures, such as bacteria that has been modified to resist antibiotic treatment. Emerging agents are previously unrecognized pathogens that might be naturally occurring and present a serious risk to human populations, such as the virus responsible for severe acute respiratory syndrome. Advanced agents are novel pathogens or other materials of biological nature that have been artificially engineered in the laboratory to bypass traditional countermeasures or produce a more severe or otherwise enhanced spectrum of disease.

medical countermeasure development and acquisition priorities in 2007, the department focused on acquisition of CBRN countermeasures using the remainder of the Project BioShield Special Reserve Fund. The plan established near-term (fiscal year 2007 through fiscal year 2008), midterm (fiscal year 2009 through fiscal year 2013), and long-term (beyond fiscal year 2013) acquisition periods to correspond with the appropriation of the funds.³⁸ Planned acquisition was based on the status of countermeasure research and development at the time. For example, the plan states that HHS's planned acquisitions for the near term were based on the availability of candidate countermeasures already in advanced development and nearing readiness for acquisition and on the ability of antibiotics already in the SNS to be used to treat the health effects of more than one biological agent.

HHS indicated in the 2007 PHEMCE plan that its updated plan would reflect its considerations of how to fund medical countermeasure development and acquisition after the funding in the Special Reserve Fund is no longer available for obligation after fiscal year 2013. The Special Reserve Fund has also been used to provide much of BARDA's advanced research and development funding, rather than annual appropriations.³⁹ HHS has indicated that it will begin a 5-year budget planning process, which could help the department consider options for funding medical countermeasure development and acquisition going forward. However, HHS has not begun to identify future funding needs for countermeasure advanced research, development, and acquisition or considered specific funding levels needed for acquiring particular countermeasures. HHS officials said that while Congress has discussed viable ways to fund countermeasure advanced research, development,

³⁸The Department of Homeland Security appropriations act for fiscal year 2004 provided that no more than \$3.4 billion of the total appropriation of approximately \$5.6 billion for the Special Reserve Fund could be obligated through fiscal year 2008, with the remainder of the funds available for obligation from fiscal year 2009 through fiscal year 2013. Pub. L. No. 108-90, 117 Stat. 1137, 1148 (2003).

³⁹Since BARDA was established in 2006, appropriations acts have transferred \$1.3 billion from the Special Reserve Fund for CBRN countermeasure basic research and advanced development, \$995 million of which has been used to fund BARDA and its advanced development contracts.

and acquisition, it was premature to anticipate congressional action on the Special Reserve Fund.⁴⁰

Although HHS has not updated the 2007 plan, DHS and HHS have continued to update their assessments of the risks that CBRN agents pose to national security and public health since the plan was released, and HHS has periodically evaluated its progress in acquiring countermeasures. DHS has conducted biennial TRAs on classes of CBRN agents and more MTAs to determine whether additional CBRN agents pose material threats.⁴¹ HHS has modeled the public health and medical consequences of some of these additional agents and established requirements for desired countermeasures for these agents, as well as more recent requirements for priority countermeasures. In addition, PHEMCE's annual reviews of the contents of the SNS have evaluated HHS's progress in acquiring countermeasures. PHEMCE conducts these annual SNS reviews to determine HHS's progress in acquiring needed countermeasures and remaining gaps, such as how many more doses of anthrax vaccine HHS may need to acquire to reach its desired goal.⁴² HHS has also conducted reviews of its medical countermeasure portfolios to identify gaps and the need for designation of additional countermeasure investment priorities. The results of the risk assessments, the PHEMCE SNS annual reviews, and the portfolio reviews could lead HHS to revise its priorities, but these reviews are not

⁴⁰Congress is currently in the process of determining the future of the Special Reserve Fund. H.R. 2405, introduced June 28, 2011, would authorize the appropriation of \$2.8 billion for the Special Reserve Fund and BARDA advanced research and development investments for fiscal year 2014 through fiscal year 2018. No more than 30 percent of the amounts authorized to be appropriated could be used for BARDA advanced research and development. As of September 2011, the Senate has not introduced any legislation pertaining to the fund. In May 2011, the Senate Committee on Health, Employment, Labor, and Pensions held a hearing on medical and public health preparedness, in which reauthorizing the Special Reserve Fund was discussed.

⁴¹Since 2007, DHS has issued six terrorism risk assessments (TRA). DHS issued biological TRAs in 2008 and 2010, chemical TRAs in 2008 and 2010, and integrated CBRN TRAs in 2008 and 2011. While DHS has issued TRAs biennially in the past, the department plans to issue them quadrennially in the future. For example, DHS plans to issue the next biological and chemical TRAs in 2014 and the next integrated CBRN TRA in 2015.

⁴²Under the requirements of the Pandemic and All-Hazards Preparedness Act, HHS is to conduct annual reviews of the SNS. HHS's first review of the SNS encompassed the years 2007 and 2008. HHS's subsequent reviews for 2009 and 2010 were finalized in 2011.

publicly available because they are sensitive. Further, these reviews do not serve the same purpose that a review and update of the PHEMCE plan would serve because they do not fully outline HHS's intentions to develop and acquire countermeasures for additional or different CBRN agents. Unlike an updated PHEMCE plan, researchers and industry partners cannot use them to determine whether they have a viable concept or candidate product that might help fulfill HHS's countermeasure needs.

Industry officials and expert groups expressed concern about the lack of specificity in the 2007 PHEMCE plan. Specifically, their concerns centered on the lack of specific information on requirements for HHS's priority countermeasures and anticipated spending to support countermeasure development. The Institute of Medicine, in its 2010 workshop summary on countermeasure development, reported that pharmaceutical and medical device manufacturing companies need additional information on how much of a countermeasure HHS wants to acquire in order to develop companies' business plans.⁴³ Industry officials also told us that they would like earlier and more specific information on requirements for desired countermeasures to guide their decisions about what products to develop. For example, one industry representative told us that pharmaceutical and medical device manufacturers do not know how many doses of a product HHS will buy, how much the company should spend on product development, and how to scale production. HHS makes information available on the desired countermeasure quantity, type, and storage requirements in requests for proposals, but some industry officials told us that it would be useful to have that information earlier.⁴⁴ The Institute of Medicine report also stated that the acquisition spending levels in the 2007 plan arbitrarily define the market, with the result that some companies will not invest in countermeasures predicted to gross less than \$100 million. Some experts have estimated the average cost of developing one countermeasure to be from \$800 million to over \$1 billion. Large companies are not likely to invest in developing a

⁴³See Institute of Medicine, *The Public Health Emergency Medical Countermeasures Enterprise: Innovative Strategies to Enhance Products from Discovery Through Approval* (Washington, D.C.: 2010).

⁴⁴Requests for proposals announce that HHS would like to award contracts to meet specific needs, such as for the development of certain quantities and formulations of a particular CBRN medical countermeasure or a particular development or manufacturing capability.

countermeasure if the perceived market is small. In addition, the Institute of Medicine report states that HHS must address long-term financing for medical countermeasures, including budgeting costs to hold or replenish these countermeasures in the SNS, to make a more rational business case for industry to invest.

Industry officials and expert groups also expressed concern about the lack of ranking within HHS's priority CBRN countermeasures. Some industry officials told us that without any further ranking, they lack assurance that their products would fulfill HHS's needs or that HHS would be committed to buying such products if their companies invested in development. Expert groups and an HHS advisory board have also reported that the lack of ranking is problematic and have made suggestions to improve the 2007 PHEMCE plan by ranking the countermeasure priorities. For example, in its 2010 report on HHS's countermeasure activities, the NBSB pointed out that HHS's countermeasure needs require further prioritization because the CBRN threats and their countermeasures are numerous.⁴⁵ As such, the NBSB recommended that HHS identify at least three high-priority, new CBRN countermeasures to develop and targeted timelines for development.⁴⁶ Similarly, the Institute of Medicine, in its 2010 report, stated that the PHEMCE plan provides limited guidance to industry and is simply a list of countermeasures that HHS hopes to acquire.⁴⁷ The report suggested revising the plan to provide more specific guidance.

HHS officials told us that the department has signaled to industry its focus on specific countermeasures for certain agents from among the larger group of priorities outlined in the 2007 plan and continues to discuss these more specific priorities with industry. As a result, HHS officials did not agree that the department needed to implement the NBSB recommendation. For example, HHS officials said that when the department began pursuing development and acquisition of countermeasures to fulfill the 2007 priorities, it focused first on acquiring

⁴⁵See National Biodefense Science Board, *Where Are the Countermeasures? Protecting America's Health from CBRN Threats* (Washington, D.C.: March 2010).

⁴⁶The National Biodefense Science Board, recommendation further stated that at least one of these three countermeasures should address radiation exposure.

⁴⁷See Institute of Medicine, *The Public Health Emergency Medical Countermeasures Enterprise: Innovative Strategies to Enhance Products from Discovery Through Approval*.

anthrax vaccine. Officials told us that once HHS acquired that vaccine for the SNS, they then focused on acquiring smallpox vaccine, and once that had been acquired, smallpox antivirals. HHS officials said they discuss HHS's countermeasure priorities during the department's annual meetings with PHEMCE stakeholders in Washington, D.C., and the department has begun holding similar meetings more recently with stakeholders in other regions of the United States. Further, HHS officials told us that the department's countermeasure priorities are very clearly articulated by the requests for proposals and other notices the department issues.

The lack of an updated plan leaves HHS without assurance that its most current needs for countermeasures to address the greatest national security and public health risks have been clearly and transparently communicated to researchers and industry partners so that the most needed countermeasures are being developed. In addition, not communicating the department's countermeasure needs in an updated plan would prolong industry's concerns about transparency and the need to have information earlier in order to develop business plans. Not providing estimates of anticipated budget priorities for developing and acquiring specific countermeasures could preclude HHS and industry from suitably targeting long-term research and development to fulfill specific countermeasure priorities, especially in tighter budget climates. The lack of specificity on spending levels could also provide further disincentives to industry to develop CBRN countermeasures in the face of developing other drugs with commercial markets that would provide a return on investment.

HHS Has Begun to Address Most of Its Own Recommendations with Initiatives Intended to Improve Medical Countermeasure Activities, but Does Not Have an Adequate Strategy to Monitor Implementation

HHS has begun to address most recommendations from its August 2010 review of its medical countermeasure activities with specific initiatives intended to improve the product development and acquisition process and PHEMCE's structure. ASPR has gathered information from HHS agencies and offices on the early stages of implementation of the initiatives that they are leading. However, the department has not developed an adequate strategy to monitor its overall implementation of the initiatives, many of which are complex in nature.

HHS Agencies and Offices Have Begun to Address Most of HHS's Recommendations with Initiatives Intended to Improve PHEMCE and Its Medical Countermeasure Activities

In HHS's August 2010 review of its medical countermeasure activities, HHS made 13 recommendations designed to improve its ability to develop and acquire medical countermeasures for novel or unknown threats, as well as countermeasures for known threats.⁴⁸ HHS's review noted that the department has continued to face challenges associated with the slow rate of progress and cost of medical countermeasure research and development. As shown in table 1, four of HHS's 13 recommendations focused on efforts to improve the infrastructure to support product development and acquisition, such as advanced development activities for, and regulatory oversight of, products, including those that can address multiple threats. The remaining nine recommendations focused on enhancing PHEMCE's structure and management, such as its decision-making processes, in order to provide pharmaceutical and medical device manufacturers with more clarity and predictability. (See app. II, table 6, for a more detailed description of the recommendations from HHS's August 2010 review and information on how HHS agencies and offices are addressing them.)

⁴⁸We examined those recommendations that were specific to CBRN countermeasure development and acquisition, and excluded those related only to pandemic influenza.

Table 1: Recommendations from HHS's August 2010 Review of PHEMCE and Its Medical Countermeasure Activities

Infrastructure to support product development and acquisition

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- | | |
|---|--|
| 1 | Enable innovative regulatory science and oversight. |
| 2 | Expand pipeline of potential products. |
| 3 | Foster partnerships to support flexible manufacturing and advanced development activities. |
| 4 | Create an independent strategic investment firm for innovation in medical countermeasures. |
-

Enhancements to PHEMCE's structure and management

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- | | |
|----|---|
| 5 | Establish a medical countermeasure development leader. |
| 6 | Establish better coordination within HHS. |
| 7 | Coordinate and collaborate with federal government partners. |
| 8 | Use a systematic approach to decision making. |
| 9 | Improve contracting and communication. |
| 10 | Improve management of product development. |
| 11 | Reexamine the statutory framework for how liability protection is provided. |
| 12 | Update the requirements for current and future products. |
| 13 | Develop multiyear budget planning process. |
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Source: GAO summary of HHS's *Public Health Emergency Medical Countermeasures Enterprise Review*.

HHS agencies and offices—primarily FDA, NIH, and BARDA—have begun to address the four recommendations to improve the infrastructure to support product development and acquisition. Specifically, HHS agencies and offices have developed initiatives intended to collectively address regulatory, technical, and business aspects of the development and acquisition process that can pose obstacles for industry and affect its ability to develop CBRN countermeasures for acquisition into the SNS. To reduce regulatory obstacles that can prevent the successful development and approval of these countermeasures, FDA has begun efforts to improve aspects of its regulatory review process; the state of regulatory science; and the legal, regulatory, and policy framework for public health response. According to some industry officials, these obstacles have included insufficient interaction with FDA officials and a lack of clarity in the regulatory process, especially when researchers are working to prove a countermeasure's effectiveness using animals as proxies for humans, as humans generally cannot ethically be used in studies involving CBRN

agents.⁴⁹ Among other things, FDA's Medical Countermeasure Initiative is intended to provide industry with greater clarity on aspects of the regulatory framework and more productive interactions with FDA regulators. For example, FDA is in the process of establishing action teams to identify resources and subject-matter experts to help identify and address scientific, regulatory, and policy issues earlier in the development process and thereby facilitate an improved regulatory review process. As of August 2011, FDA officials told us they have established three action teams, including one focused on laboratory diagnostic tests.

To reduce technical obstacles affecting the number of potential products in the research and development pipeline, NIH has begun to implement the Concept Acceleration Program. This program would proactively seek out and provide additional resources to investigators who are conducting promising basic research with the potential to fulfill a particular CBRN medical countermeasure need. NIH officials told us they have begun hiring staff for this program and hope to complete hiring by the end of 2011.

To reduce technical and business obstacles that can affect the probability of successfully developing technologies and countermeasures, BARDA has begun to address two of the recommendations intended to provide expertise and resources to pharmaceutical and medical device manufacturers with limited experience or knowledge of the technical and business aspects of product development. First, to assist small companies that have limited technical experience with advanced development and flexible manufacturing services, BARDA has begun to implement its plan to open Centers for Innovation in Advanced Development and Manufacturing. The centers are intended to provide industry with technical expertise and services in scale-up manufacturing, regulatory affairs, and other aspects of advanced development and manufacturing. BARDA issued a request for proposals for these centers in March 2011 with the expectation that the first centers would open in 2014. Second, to assist small companies with limited experience in a

⁴⁹We have previously reported on challenges with regulatory processes for evaluating promising medical countermeasures. In addition to challenges in proving a countermeasure's effectiveness using animals as proxies for humans, other challenges include (1) determining appropriate doses of countermeasures for children, who may be more vulnerable to exposure to CBRN agents, and (2) evaluating the safety and effectiveness of medical countermeasures for use in a public health emergency if they have not yet been FDA-approved or licensed. See [GAO-11-567T](#).

range of business and financing issues, BARDA has developed plans for a Medical Countermeasure Strategic Investor. This independent investment firm would identify companies with technologies that are both likely to result in a commercially attractive product and capable of producing medical countermeasures that satisfy government-specific needs. The investment firm would assist these companies with capital and business expertise as they navigate the countermeasure development process and become eligible for advanced research and development funding. HHS has sought statutory authority to create the investment firm and has developed various options for how to structure it.

HHS agencies and offices—especially ASPR—have begun steps intended to address many of the nine recommendations to enhance PHEMCE’s structure and management but have not taken steps to address all of them. (See app. II, table 6, for more information on how HHS agencies and offices are addressing each of the recommendations from HHS’s August 2010 review of PHEMCE and its medical countermeasure activities.) For example, HHS has taken the following actions:

- In response to two recommendations in the August 2010 review—to improve coordination within HHS and with federal government partners—ASPR created the Enterprise Senior Council (ESC) as the decision forum for PHEMCE. According to HHS officials, the ESC, unlike its predecessor, includes voting members representing each federal department that participates in PHEMCE.⁵⁰ HHS officials also said that some officials who have participated in the ESC have been at more senior levels within their departments than the officials who served on the ESC’s predecessor.
- To improve management of countermeasure development—as recommended in HHS’s August 2010 review—HHS agencies and offices, through ASPR, have jointly conducted portfolio reviews of all of HHS’s investments in countermeasures to address a given agent (including anthrax, smallpox, and radiological and nuclear agents). HHS officials told us that during these reviews, each agency presented its perspective on the critical issues in developing

⁵⁰These departments include DHS, DOD, the Department of Agriculture, and the Department of Veterans Affairs. The ESC’s predecessor included only HHS agencies among its voting members.

countermeasures for that agent. Officials said that these reviews have allowed senior leaders from across HHS to better understand the range of available countermeasures for each threat and highlight any gaps or issues in countermeasure development or response capabilities. HHS developed a list of key challenges within and across the portfolios and assigned responsibility for addressing those challenges to the appropriate agencies and offices.

- HHS has begun to develop a 5-year budget planning process, as recommended in HHS's August 2010 review. This planning process, which would not replace the annual budget and appropriations process, would help the department determine how to fund medical countermeasures as they move through development, acquisition, and stockpile replenishment and as responsibility for the countermeasures moves from one HHS agency or office to another. Some industry officials we spoke with told us that such a process would increase their confidence in HHS by demonstrating, for example, that the department has thought through the funding for the whole lifecycle of a product, including replenishment of the product once it is in the SNS.

HHS has not taken steps to address one of the recommendations from its August 2010 review. As of August 2011, HHS officials said that they have not appointed a medical countermeasure development leader with the sole job of coordinating and integrating the multiple HHS medical countermeasure development activities, as recommended in the review. Officials said that the ESC has instead acted in this capacity.

HHS Has Gathered Information on Agencies' and Offices' Implementation of the Initiatives but Has Not Developed an Adequate Strategy to Monitor Overall Progress

HHS has gathered information on agencies' and offices' implementation of its initiatives from the August 2010 review intended to improve PHEMCE and the department's medical countermeasure activities. With ASPR as the lead agency responsible for monitoring implementation, ASPR officials have gathered information from the responsible HHS agencies and offices on the individual initiatives. This information, which ASPR officials have compiled into an overall planning document, includes planned time frames and milestones, status of any funding necessary to implement initiatives, potential barriers to implementation, and possible options for mitigating these barriers. ASPR officials said that they intend to request quarterly updates to revise the planning document.

Although HHS has compiled a planning document and conducted periodic reviews of progress, HHS has not developed an adequate strategy that meets federal internal control standards and best practices for program management for monitoring the overall progress of the initiatives. Federal internal control standards and best practices for program management indicate the importance of a strategy to monitor implementation. Federal internal control standards call for agencies to review and evaluate actual performance against planned or expected results on an ongoing basis and determine proper actions to address identified differences.⁵¹ Best practices for program management call for a centralized and coordinated strategy to align efforts between the multiple projects that make up a program and to monitor the overall progress of the program.⁵² According to HHS officials, their monitoring strategy calls for quarterly updates of ASPR's planning document, a high-level annual review of progress by senior HHS officials, and more focused quarterly reviews by the ESC. However, it is not clear that these activities will provide ASPR or the ESC with the ongoing information needed to centrally and actively monitor the agencies' and offices' overall implementation of the initiatives, completion of any associated tasks or activities, and resolution of any deficiencies. For example:

- As of June 2011, ASPR's planning document did not contain complete information for each initiative that could be used by ASPR officials for monitoring purposes. For example, the document does not have information on planned time frames and milestones for four initiatives, including the initiatives to improve management of product development and develop a multiyear budget planning process.
- As of June 2011, the planning document did not portray information in a way that makes it easy for ASPR officials to clearly identify progress or gaps in implementation across all of the initiatives.
- Various HHS and PHEMCE entities have responsibility for monitoring and oversight of these initiatives, including conducting the quarterly and annual reviews, but it is not clear how monitoring and implementation activities are centrally coordinated throughout the department. In August 2011, HHS officials told us ASPR is the lead

⁵¹GAO/AIMD-00-21.3.1.

⁵²Project Management Institute, *The Standard for Program Management*.

agency for monitoring overall implementation of the initiatives and, as such, designated a senior official to maintain the planning document. However, the various agencies and offices implementing the initiatives are responsible for any coordination and partnerships that are needed to implement their respective initiatives.

- Even if HHS had clear and coordinated leadership, HHS does not actively monitor the agencies' and offices' progress in implementing the initiatives or their resolution of any deficiencies. ASPR sends requests for status updates to the responsible office or agency. Therefore, the information in the planning document depends upon the completeness of the information that ASPR receives. In addition, ASPR officials told us that the various agencies and offices implementing the initiatives—and not ASPR—are responsible for bringing any issues or deficiencies in implementation to the attention of the ESC during the quarterly reviews.

Because HHS does not have an adequate strategy to monitor HHS agencies' and offices' ongoing implementation of their initiatives that meets federal internal control standards and best practices for program management, the department lacks assurance that implementation of the initiatives is coordinated across HHS and PHEMCE in order to improve HHS's ability to develop and acquire medical countermeasures.

HHS's PHEMCE and Related Written Agreements Have Facilitated Intradepartmental Coordination, but Challenges Remain That May Be Addressed through HHS's New Initiatives

HHS's establishment of PHEMCE in 2006 and its subsequent written agreements have facilitated intradepartmental coordination on the development and acquisition of medical countermeasures, but some coordination challenges remain that may be addressed by HHS's new initiatives. Features that have facilitated intradepartmental coordination include establishing an agreed-upon collaboration process through PHEMCE and documenting the agreements on the roles and responsibilities of PHEMCE's intradepartmental partners. However, industry and outside experts have reported that HHS's agencies and offices lack internal coordination in the development and acquisition of medical countermeasures, which hampers industry's ability to develop needed CBRN countermeasures. Implementing the initiatives from HHS's August 2010 review may mitigate some of these coordination challenges.

HHS's PHEMCE Structure and Related Written Agreements Have Facilitated Intradepartmental Coordination

Since 2006, PHEMCE has provided a structure for improved coordination among HHS's agencies and offices, including ASPR, BARDA, CDC, FDA, and NIH. HHS officials told us that before PHEMCE was established, CDC had limited information about what medical countermeasures were being developed and faced challenges in working with BARDA on setting requirements for medical countermeasures for the SNS. CDC officials indicated that since PHEMCE was established, coordination with BARDA has improved. Further, officials from NIH told us that through PHEMCE, NIH has coordinated with other HHS agencies to move medical countermeasures from the early development phase into the advanced development phase at BARDA. For example, NIH helped move a smallpox antiviral from a support and guidance phase at NIH to BARDA for advanced development and acquisition. In addition, NIH officials said that for some CBRN agents, FDA has been proactive in supporting medical countermeasure development. For example, FDA has helped to identify suitable animal models or other tools necessary for its regulatory review process.

Further, the establishment of related written agreements—a PHEMCE memorandum of understanding (MOU) and charters for PHEMCE's working groups—reinforced intradepartmental coordination and collaboration on efforts to support medical countermeasure development and acquisition by establishing areas of responsibility. The PHEMCE MOU and the working group charters described the roles and responsibilities for each HHS agency and office with respect to the development and acquisition of medical countermeasures, and stipulated the minimum meeting frequency of the PHEMCE governing body and working groups. Consistent with our best practices for enhancing and sustaining coordination,⁵³ these written agreements served as guidance for HHS's agencies and offices, clarifying the roles of the various agencies, whose missions often have conflicting objectives that reflect different aspects of complex public problems. The written agreements also facilitated clear lines of responsibility and accountability for crosscutting program efforts.⁵⁴

⁵³GAO-06-15 and GAO/AIMD-00-21.3.1.

⁵⁴See GAO, *National Preparedness: DHS and HHS Can Further Strengthen Coordination for Chemical, Biological, Radiological, and Nuclear Risk Assessments*, GAO-11-606 (Washington, D.C.: June 21, 2011).

These agreements have lapsed or become outdated, but HHS is working to renew them. The PHEMCE intradepartmental MOU expired in July 2010. In August 2011, HHS officials told us that a new MOU had been drafted and was being routed through the various HHS agencies and offices for approval and signature. Similarly, the original PHEMCE governing body's charter has become outdated due to changes in PHEMCE membership and the establishment of the ESC. HHS officials also told us that a new charter for the ESC would enhance and sustain intradepartmental coordination on countermeasure development and acquisition activities. HHS drafted the ESC charter to incorporate modifications made to the PHEMCE governing body. As of August 2011, the charter had been routed through the department for approval and signature and had been sent to the other federal department partners for approval and signature.

Industry and Outside Experts Indicate That New Initiatives May Mitigate Some Coordination Challenges

Although PHEMCE and the related written agreements have facilitated intradepartmental coordination, industry officials and outside experts that we spoke with said that certain challenges in coordination have continued. They indicated that the challenges were due primarily to inconsistent procedures for coordination and unclear roles and responsibilities of each of HHS's agencies in advancing products along HHS's product development and acquisition pipeline. According to our best practices, agencies can enhance and sustain their coordination by adopting key practices—including defining and articulating a common outcome, agreeing on roles and responsibilities, and establishing compatible policies and procedures for operating across agency boundaries.⁵⁵ Comprehensive written agreements, such as the PHEMCE MOU and related charters, can help clarify roles, responsibilities, policies, and procedures. Industry officials stated that the roles and responsibilities of each of HHS's agencies and offices in coordinating to advance products along HHS's product development and acquisition pipeline are not transparent, thus hampering industry's ability to develop CBRN countermeasures.

Inconsistent procedures and a lack of clarity on the roles and responsibilities of each HHS agency or office in moving CBRN countermeasures through HHS's development pipeline can slow the

⁵⁵[GAO-06-15](#) and [GAO/AIMD-00-21.3.1](#).

already lengthy and complex development process for acquiring products. In its 2010 report on HHS's countermeasure activities, the NBSB reported that there has been insufficient coordination among HHS's agencies and offices to successfully develop and acquire medical countermeasures.⁵⁶ For example, the NBSB reported that NIH and BARDA needed to coordinate the transition of products between the two agencies so that the evaluation of promising candidates can proceed effectively. Similarly, in its 2010 countermeasure development workshop summary, the Institute of Medicine reported that the process to acquire countermeasures for the SNS is perceived by industry to be lengthy, opaque, and unpredictable.⁵⁷ In particular, the process to transition countermeasures from advanced development to acquisition is unclear.

Industry officials told us that FDA, NIH, and BARDA have not always coordinated effectively to communicate regulatory requirements throughout the development process for CBRN medical countermeasures. Some industry officials told us that companies must work with both NIH and FDA during the early development stage to ensure that they are adhering to good manufacturing practices and to anticipate and help prevent any problems in obtaining future FDA approval or licensure after countermeasures are delivered to the SNS. In addition, BARDA is to work with companies during the advanced development stage to prepare them for the FDA approval process. However, there is no established mechanism for ensuring that these interactions occur between the companies, FDA, NIH, and BARDA. In addition, some industry officials told us that BARDA and FDA have not coordinated effectively to reduce uncertainties associated with the FDA regulatory process in the advanced development stage. The officials said that, for example, one company had worked closely with BARDA throughout the development process to determine information needed for countermeasure development but that when the company applied for FDA approval for its countermeasure, FDA asked for information that BARDA had not discussed with the company, and therefore, the company did not anticipate having to provide. When the company could not supply the information, FDA withheld approval. According to HHS, it is common

⁵⁶See National Biodefense Science Board, *Where Are the Countermeasures? Protecting America's Health from CBRN Threats*.

⁵⁷See Institute of Medicine, *The Public Health Emergency Medical Countermeasures Enterprise: Innovative Strategies to Enhance Products from Discovery Through Approval*.

practice for FDA to request additional information from pharmaceutical companies that had not been disclosed in previous discussions regarding safety and efficacy testing of their products. HHS officials told us that BARDA has expanded its outreach to FDA to address specific requirements, resolve problems, and help move products toward approval.

Furthermore, industry officials from several companies also indicated that roles and responsibilities for BARDA and CDC to move countermeasures into the SNS have not always been clear. CDC is responsible for maintaining the SNS, and CDC officials told us that they develop and update a 5-year project plan for each countermeasure in the SNS for shelf life, storage and space requirements, and other specific needs. Despite this role, industry officials from several companies indicated that CDC was not brought into the process early enough in product development. For example, industry officials from one company told us that CDC had not been involved in the development process when their company was making decisions regarding product characteristics, such as refrigeration or multi-dose or single-dose vials. According to these officials, if CDC had been involved earlier, the company might have developed the product with different characteristics that CDC might consider improvements for dispensing. Industry officials from another company said that CDC officials were not familiar with the product characteristics of a new countermeasure that they had developed for delivery to the SNS—even though BARDA officials were familiar with their product. These industry officials indicated that more interaction with CDC earlier in the process could improve countermeasure development. CDC officials stated that they were involved in the development of product requirements.

HHS officials and some industry officials we spoke with expect that some of the new initiatives may help reinforce coordination among HHS's agencies for developing and acquiring medical countermeasures. However, it is too early to tell whether the initiatives will improve these challenges. Examples of initiatives that may reinforce coordination include the following:

- The creation of the ESC as PHEMCE's decision forum represents HHS's effort to build cohesiveness within the PHEMCE structure by providing a stronger governing role and by ensuring that the agencies' senior leaders participate in the medical countermeasure decision-making process.

-
- The medical countermeasure development leader, if created, could help solve challenges related to coordinating HHS's multiple countermeasure development activities and working with HHS's agencies that have their own missions and infrastructures.
 - ASPR's portfolio reviews, intended to provide overviews and updates on the range of medical countermeasure activities for specific CBRN agents, could further facilitate intradepartmental coordination. During these reviews, senior leaders from each HHS agency come together to discuss critical issues in developing countermeasures for these agents (including anthrax, smallpox, and radiological and nuclear agents). HHS officials told us that during these reviews, agency officials identified issues that needed to be resolved to move forward with development of countermeasures and worked together to determine specific actions HHS needed to take to address these issues.

Conclusions

Given the complexity and importance of developing and acquiring medical countermeasures, HHS would benefit from updating its countermeasure plan to indicate whether there have been any changes in priorities and monitoring implementation of its new initiatives to address its own recommendations. Establishing a new or revised PHEMCE implementation plan for countermeasure development and acquisition that includes planned funding for these activities in the coming years could set the stage for a more transparent, integrated, and focused dialogue between HHS and its federal and industry partners. Including more specific information on anticipated budget priorities for countermeasure acquisition would also be helpful to encourage industry to invest in countermeasure development. Further, as the HHS agencies and offices begin to implement initiatives to improve PHEMCE and HHS's medical countermeasure activities, the department could benefit by developing a strategy to monitor the overall progress of the initiatives that meets federal internal control standards and best practices for program management. Although various HHS agencies and offices have the lead in implementing the different initiatives, virtually all of HHS's initiatives require the concerted and coordinated efforts of its intradepartmental agencies. Without an adequate monitoring strategy, HHS will be unable to track its overall progress in implementing the initiatives and hold its agencies and offices accountable for improving its emergency medical countermeasure activities. One key aspect of HHS's PHEMCE review was to improve coordination within HHS. By implementing the initiatives to improve coordination and renewing its written agreements, HHS is working to ensure that its agencies and offices reach concurrence on their

expected levels of involvement and responsibility at each stage of the countermeasure development and acquisition process.

Recommendations for Executive Action

We are making three recommendations to improve HHS's countermeasure enterprise and activities.

To ensure HHS's stated medical countermeasure development and acquisition priorities are based on current risk assessments for CBRN agents and the status of recent countermeasure research and development and are clearly and transparently communicated, we recommend that the Secretary of HHS take the following two actions:

- update the PHEMCE implementation plan for CBRN medical countermeasure development and acquisition, and
- incorporate into the updated plan more specific information on anticipated budget priorities for countermeasure acquisition.

To provide reasonable assurance that HHS's initiatives will achieve the intended results of improving PHEMCE and its medical countermeasure activities, we recommend that the Secretary of HHS develop a strategy to monitor the overall progress and results of implementing the initiatives by obtaining complete information on all initiatives.

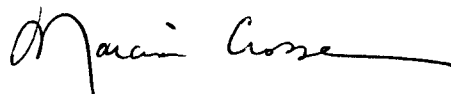
Agency Comments and Our Evaluation

We provided a draft of this report to HHS, and its comments are reprinted in appendix III. In its comments, HHS agreed with two of our recommendations and provided information related to the third one. HHS concurred with the first recommendation to update the PHEMCE implementation plan. Consistent with our second recommendation to incorporate more specific information on anticipated budget priorities for countermeasure acquisition into the updated plan, HHS agreed that one critical issue for the updated PHEMCE plan was how best to communicate information on anticipated spending levels for the highest-priority medical countermeasures. For the third recommendation on having a strategy to monitor the overall progress and results of implementing the initiatives to address its own recommendations, HHS stated in its September 2011 comments that one of the overarching PHEMCE accomplishments was having developed such a strategy and provided us with an outline of the strategy. However, as of August 2011, HHS had not provided us with any evidence of such a strategy. As we state in the report, the strategy for monitoring implementation of its

initiatives that HHS outlined in its comments does not meet federal internal control standards and best practices for program management. HHS's planning document did not contain complete information for each initiative and did not portray information in a way that would facilitate understanding of progress or gaps in implementation across the initiatives as of June 2011, the latest quarter for which documentation was available. As late as June 2011, HHS officials told us that no individual or office was designated as responsible for actively monitoring implementation across the initiatives. Further, the HHS agencies and offices responsible for implementing the initiatives are also responsible for bringing any issues or deficiencies in implementation to the attention of the ESC. Therefore, we maintain that HHS needs to develop a monitoring strategy that involves obtaining complete information on all the initiatives, including planned time frames and milestones, and to actively assess progress across all the initiatives to ensure that management's directives are achieved.

HHS's comments also provided information on PHEMCE and its actions and agency contributions to PHEMCE efforts. For example, HHS provided information on activities of PHEMCE, BARDA, FDA, CDC, and NIH, information that it characterized as high-level snapshots of activities under way that reflect the significant and ongoing investment of energy and resources for medical countermeasure research, development, acquisition, procurement, and distribution. In addition, HHS provided technical comments, which we incorporated as appropriate.

We are sending copies of this report to the Secretary of HHS and to interested congressional committees. The report is also available at no charge on the GAO Web site at <http://www.gao.gov>. If you or your staffs have any questions about this report, please contact me at (202) 512-7114 or crosse@gao.gov. Contact points for our Offices of Congressional Relations and Public Affairs may be found on the last page of this report. Key contributors to this report are listed in appendix IV.



Marcia Crosse
Director, Health Care

Appendix I: HHS Medical Countermeasure Development and Acquisition Priorities and Investments

Since 2007, the Department of Health and Human Services (HHS) has invested approximately \$4.3 billion in medical countermeasure research, development, and acquisition for chemical, biological, radiological, and nuclear (CBRN) agents.¹ HHS has sought to develop and acquire particular medical countermeasures for the CBRN agents that the Department of Homeland Security (DHS) deemed material threats to the nation and for an additional chemical agent that DHS has not declared a material threat, based on DHS's material threat assessments (MTA) and HHS's public health consequence modeling reports.² HHS published its CBRN medical countermeasure priorities in the 2007 Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) implementation plan, which included three time frames for planned acquisitions based on the timing of the availability of funds from the Project BioShield Special Reserve Fund and the status of countermeasure research and development at the time. (See table 2 for HHS's countermeasure priorities and time frames from the 2007 plan.)

¹For the purposes of this report, we used the term "invested" to mean obligated.

²See Department of Health and Human Services, Assistant Secretary for Preparedness and Response, *HHS Public Health Emergency Medical Countermeasure Enterprise Implementation Plan for Chemical, Biological, Radiological and Nuclear Threats* (Washington, D.C.: April 2007).

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Table 2: HHS’s Medical Countermeasure Priorities and Time Frames by Fiscal Year, as of April 2007

HHS CBRN medical countermeasure priorities	Near term (FY 2007 – FY 2008)	Midterm (FY 2009 – FY 2013)	Long term (beyond FY 2013)
Anthrax antitoxin		X	
Anthrax vaccine	X		
Anthrax broad spectrum antibiotics	X	X	
Anthrax diagnostics		X	
Botulism diagnostics		X	
Ebola/Marburg broad spectrum antivirals			X
Ebola/Marburg countermeasures		X	
Ebola/Marburg diagnostics		X	
Glanders/melioidosis broad spectrum antibiotics	X	X	
Glanders/melioidosis diagnostics		X	
Junin broad spectrum antivirals			X
Junin diagnostics		X	
Plague broad spectrum antibiotics	X	X	
Plague diagnostics		X	
Radiological and nuclear, acute radiation syndrome/delayed effects of acute radiation exposure countermeasures	X	X	
Radiological and nuclear biodosimetry/bioassay (diagnostics)		X	
Radionuclide-specific agents		X	
Smallpox broad spectrum antivirals			X
Smallpox diagnostics		X	
Smallpox antiviral		X	
Smallpox vaccine	X		
Tularemia broad spectrum antibiotics	X	X	
Tularemia diagnostics		X	
Typhus broad spectrum antibiotics	X	X	
Typhus diagnostics		X	
Chemical/volatile nerve agent single antidote			X
Chemical/Enterprise CHEMPACKs		X	

Source: GAO analysis of HHS Public Health Emergency Medical Countermeasure Enterprise Implementation Plan for Chemical, Biological, Radiological and Nuclear Threats, April 2007.

Because HHS has not been able to immediately acquire some countermeasures listed as priorities in the 2007 PHEMCE plan, HHS has invested approximately \$1.9 billion in research and development from fiscal year 2007 through fiscal year 2010. Of this \$1.9 billion, the National Institutes of Health (NIH) invested \$1.2 billion in early research and early development, and the Biomedical Advanced Research and Development Authority (BARDA) invested \$681 million in advanced research and development. (See tables 3 and 4 for NIH and BARDA investments, respectively.) From fiscal year 2007 through fiscal year 2010, HHS spent \$2.4 billion to acquire available countermeasures for the Strategic National Stockpile (SNS). Of this \$2.4 billion, HHS invested \$1.1 billion of the Special Reserve Fund to acquire countermeasures for the SNS, and the Centers for Disease Control and Prevention (CDC) invested \$1.3 billion to maintain quantities of countermeasures held in the SNS. (See table 5 for HHS Project BioShield Special Reserve Fund investments.)

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Table 3: NIH Investments in CBRN Medical Countermeasure Early Research and Development, Fiscal Year 2007 through Fiscal Year 2010

Disease/agent	Medical countermeasure	FY 2007 investments	FY 2008 investments	FY 2009 investments ^a	FY 2010 investments ^a	Totals
Anthrax (<i>Bacillus anthracis</i>) ^b	Diagnostics	\$4,227,137	\$1,496,177	\$3,669,816	\$2,683,865	\$12,076,995
	Therapeutics (anthrax antitoxin) ^c	39,747,858	28,154,770	33,385,361	27,076,406	128,364,395
	Vaccine	26,014,877	32,346,017	16,457,633	44,778,727	119,597,254
Total, anthrax		69,989,872	61,996,964	53,512,810	74,538,998	260,038,644
Botulism toxin (<i>Clostridium botulinum</i>)	Diagnostics	1,894,757	380,915	2,131,347	2,175,551	6,582,570
Total, botulism toxin		1,894,757	380,915	2,131,347	2,175,551	6,582,570
Ebola and Marburg (viral hemorrhagic fevers)	Antivirals (Ebola)	3,066,405	11,602,269	8,130,700	8,853,362	31,652,736
	Antivirals (Marburg)	797,157	532,309	2,847,050	2,240,903	6,417,419
	Diagnostics (Ebola)	—	145,194	196,963	1,281,618	1,623,775
	Diagnostics (Marburg)	—	145,194	121,320	176,057	442,571
	Vaccine (Ebola)	31,180,016	30,586,592	21,653,115	37,163,860	120,583,583
Vaccine (Marburg)	10,962,249	18,580,539	15,137,164	26,822,113	71,502,065	
Total, Ebola and Marburg		46,005,827	61,592,097	48,086,312	76,537,913	232,222,149
Glanders/melioidosis (<i>Burkholderia</i>)	Diagnostics	1,044,626	1,675,818	3,383,319	3,437,768	9,541,531
Total, glanders and melioidosis		1,044,626	1,675,818	3,383,319	3,437,768	9,541,531
Junin (viral hemorrhagic fever)	Diagnostics	55,477	44,176	32,601	67,080	199,334
Total, Junin		55,477	44,176	32,601	67,080	199,334
Plague (<i>Yersinia pestis</i>)	Diagnostics	754,835	738,778	1,607,449	1,392,686	4,493,748
Total, plague		754,835	738,778	1,607,449	1,392,686	4,493,748
Smallpox (<i>Variola major</i>) ^d	Diagnostics	1,068,331	2,145,659	2,179,066	3,798,182	9,191,238
	Therapeutics (smallpox antiviral)	32,264,000	21,983,318	32,532,188	24,041,364	110,820,870
	Vaccine	55,650,473	58,558,339	26,568,676	34,189,768	174,967,256
Total, smallpox		88,982,804	82,687,316	61,279,930	62,029,314	294,979,364
Tularemia (<i>Francisella tularensis</i>)	Diagnostics	2,788,482	33,939	1,361,838	1,360,410	5,544,669

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Disease/agent	Medical countermeasure	FY 2007 investments	FY 2008 investments	FY 2009 investments^a	FY 2010 investments^a	Totals
Total, tularemia		2,788,482	33,939	1,361,838	1,360,410	5,544,669
Typhus (Rickettsia prowazekii)	Diagnostics	—	222,387	169,731	181,923	574,041
Total, typhus		—	222,387	169,731	181,923	574,041
Chemical		49,542,368	48,651,701	49,138,476	49,847,755	197,180,300
Total, chemical		49,542,368	48,651,701	49,138,476	49,847,755	197,180,300
Radiological and nuclear		46,487,561	45,700,000	47,571,244	46,858,859	186,617,664
Total, radiological and nuclear		46,487,561	45,700,000	47,571,244	46,858,859	186,617,664
Totals		307,546,609	303,724,091	268,275,057	318,428,257	1,197,974,014

Source: GAO analysis of NIH data.

Note: NIH does not break out its investments in early research and development of radiological, nuclear, and chemical agents by type of countermeasure. Also, in addition to NIH investments in the specific countermeasures HHS identified as priorities, NIH has invested in research related to other countermeasures for the 14 agents in the 2007 PHEMCE plan. For example, HHS's only priority countermeasure identified to address botulinum toxin was for diagnostics, but NIH also invested in research on countermeasures to treat the effects of the toxin on affected individuals. NIH's investments also include research on other potential public health threats, including foodborne diseases such as salmonella, emerging infectious diseases such as severe acute respiratory syndrome, and other agents such as ricin toxin.

^aNIH CBRN medical countermeasure investments for fiscal years 2009 and 2010 include the use of funds from the American Recovery and Reinvestment Act of 2009. Pub. L. No. 111-5, 123 Stat. 115 (2009).

^bNIH investments in countermeasures for anthrax include multidrug-resistant anthrax.

^cNIH investments in anthrax therapeutics may also include antibiotics for preventing infection after exposure, in addition to treating the health effects of infection with anthrax.

^dNIH investments in countermeasures for smallpox include other pox viruses, such as monkeypox.

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Table 4: BARDA Investments in CBRN Medical Countermeasure Advanced Research and Development, Fiscal Year 2007 through Fiscal Year 2010

Medical countermeasure	FY 2007 investments	FY 2008 investments	FY 2009 investments	FY 2010 investments	Totals
Anthrax therapeutics	\$27,080,000	\$12,750,000	\$31,430,000	\$43,400,000	\$114,660,000
Anthrax vaccines	43,420,000	13,070,000	81,370,000	105,660,000	243,520,000
Broad spectrum antibiotics	9,000,000	1,050,000	4,900,000	27,560,000	42,510,000
Smallpox medical countermeasures	2,980,000	18,070,000	21,000,000	11,300,000	53,350,000
Radiological and nuclear medical countermeasures	10,000,000	29,860,000	67,190,000	38,950,000	146,000,000
Biodosimetry (radiological and nuclear) ^a	—	—	36,050,000	1,930,000	37,980,000
Chemical medical countermeasures	6,320,000	700,000	2,000,000	2,100,000	11,120,000
Innovation ^b	—	—	7,000,000	24,680,000	31,680,000
Totals	98,800,000	75,500,000	250,940,000	255,580,000	680,820,000

Source: GAO analysis of BARDA data.

^aBiodosimetry, including radiation bioassays, are diagnostic tools to determine the level of radiation and the type of radioactive isotope to which an individual is exposed.

^bInnovation includes investments in tools to enhance individuals' immune response to vaccines, stabilize vaccines, and other tools.

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Table 5: HHS Investments in CBRN Medical Countermeasures Using the Project BioShield Special Reserve Fund, Fiscal Year 2007 through Fiscal Year 2010

Medical countermeasure	Project BioShield investment FY 2007	Project BioShield investment FY 2008	Project BioShield investment FY 2009	Project BioShield investment FY 2010	Totals
Anthrax antitoxin	—	—	\$152,000,000	—	\$152,000,000
Anthrax vaccine	448,000,000	—	—	—	448,000,000
Smallpox vaccine	505,000,000	—	—	—	505,000,000
Totals	953,000,000	—	152,000,000	—	1,105,000,000

Source: GAO analysis of HHS information.

Note: From 2004 through 2006, HHS also invested about \$1 billion of the Project BioShield Special Reserve Fund to acquire certain medical countermeasures. Specifically, HHS invested \$318 million to acquire anthrax antitoxin, \$245 million to acquire anthrax vaccine, \$415 million to acquire botulinum antitoxin, and \$40 million to acquire a pediatric formulation of potassium iodide and calcium and zinc diethylene triamine penta-acetic acid—countermeasures that block the absorption of radioactive iodine or remove radiation from the body.

Appendix II: HHS Initiatives to Address Its Recommendations to Improve Medical Countermeasure Activities

In 2006, the Department of Health and Human Services (HHS) established the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE), a federal interagency body responsible for providing recommendations to the Secretary of HHS on the development and acquisition of chemical, biological, radiological, and nuclear (CBRN) medical countermeasures, among other responsibilities.¹ In December 2009, the HHS Secretary called for a comprehensive review of PHEMCE and the department's medical countermeasure development and acquisition activities. As a result, HHS issued an August 2010 review with 13 recommendations to improve its infrastructure to support product development and acquisition and to enhance PHEMCE's structure and management.² Table 6 shows the 13 recommendations, HHS's initiatives to address the recommendations, the HHS lead organization that is responsible for implementation, and HHS's reported actions to implement the initiatives.

¹PHEMCE is composed primarily of officials from HHS's Office of the Assistant Secretary for Preparedness and Response (ASPR), the Biomedical Advanced Research and Development Authority (BARDA), the Centers for Disease Control and Prevention (CDC), the Food and Drug Administration (FDA), and the National Institutes of Health (NIH). In addition, PHEMCE includes officials from the Department of Homeland Security, the Department of Defense, the Department of Veterans Affairs, the Department of Agriculture, and the Executive Office of the President.

²See Department of Health and Human Services, Assistant Secretary for Preparedness and Response, *The Public Health Emergency Medical Countermeasures Enterprise Review: Transforming the Enterprise to Meet Long-Range National Needs* (Washington, D.C.: August 2010). We examined only those recommendations that were specific to CBRN countermeasures and not those for pandemic influenza.

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Table 6: Recommendations from HHS’s August 2010 Review of PHEMCE and Its Medical Countermeasure Activities and HHS’s Initiatives in Response

#	Recommendation from HHS’s August 2010 Review	HHS’s Initiative to Address Recommendation (lead organization)	Reported Actions to Implement Initiatives
Recommendations and Initiatives on Infrastructure to Support Product Development and Acquisition			
1	Enable innovative regulatory science and oversight.	Medical Countermeasure Initiative to provide developers of medical countermeasures with greater clarity on aspects of the regulatory framework and more interaction with FDA regulators by (1) enhancing the review process by establishing action teams; (2) advancing regulatory science; and (3) modernizing the legal, regulatory, and policy framework for public health response (FDA).	FDA officials reported taking several actions to address the recommendations, including establishing action teams to identify resources and subject-matter expertise to facilitate an improved regulatory review process for different products and developing statutory proposals that could improve public health response.
2	Expand pipeline of potential products.	Concept Acceleration Program to increase the number of potential products in the pipeline by proactively seeking out and providing additional resources to investigators who are conducting promising basic research with the potential to fulfill a particular CBRN medical countermeasure need (NIH).	NIH officials said they have begun hiring staff for this program and hope to complete hiring by the end of 2011.
3	Foster partnerships to support flexible manufacturing and advanced development activities.	Centers for Innovation in Advanced Development and Manufacturing to provide companies with technical expertise and services in scale-up manufacturing, regulatory affairs, and other aspects of advanced development and manufacturing (BARDA).	BARDA issued a request for proposals in March 2011, with a June 2011 deadline for responses. BARDA officials anticipate that the first centers will open in 2014.
4	Create an independent strategic investment firm for innovation in medical countermeasures.	Strategic Investor Initiative to identify and provide capital and business expertise to companies with technologies that are both likely to result in a commercially attractive product and capable of producing medical countermeasures that satisfy government-specific needs (BARDA).	BARDA has sought statutory authority from Congress to create the investment firm and has developed various options for how to structure it.
Recommendations and Initiatives on Enhancements to PHEMCE’s Structure and Management			
5	Establish a medical countermeasure development leader.	Medical countermeasure development leader, independent of the ASPR, to coordinate and integrate the multiple efforts and programs within HHS to assist in successful development of medical countermeasures (HHS Office of the Secretary).	HHS officials said that they have not appointed a medical countermeasure development leader and that the Enterprise Senior Council (ESC) is acting in this capacity.
6	Establish better coordination within HHS.	Establishment of ESC as the decision forum for PHEMCE with expanded representation by non-HHS partners (ASPR).	HHS officials said the HHS agencies and offices have signed the charter to create the ESC, which first met in February 2011.
7	Coordinate and collaborate with federal government partners.		
8	Use a systematic approach to decision making.	Development of a systematic approach for analyzing the considerations and trade-offs in decisions throughout the process of developing and acquiring medical countermeasures (ASPR).	ASPR has approved the use of a decision management system that can be used to assess competing investments in medical countermeasures based on economic and other decision analysis tools.

**Appendix II: HHS Initiatives to Address Its
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#	Recommendation from HHS's August 2010 Review	HHS's Initiative to Address Recommendation (lead organization)	Reported Actions to Implement Initiatives
9	Improve contracting and communication.	Methods to improve speed of contracting and decision-making processes and development of HHS-level policy on use of Other Transaction Authorities, which are mechanisms other than contracts, grants, and cooperative agreements that can provide more flexible, faster ways of procuring goods and services (ASPR, BARDA, NIH).	A team of ASPR and BARDA contracting officials and scientists reviewed the contracting process and recommended potential actions and new procurement methods to improve the speed of the process. In addition, HHS officials reported that they have provided ASPR employees with additional training on communicating with companies during the contracting process, and HHS officials have begun holding meetings with industry stakeholders in different regions of the United States.
10	Improve management of product development.	Portfolio reviews across all of HHS's investments for countermeasures for addressing a given agent to better understand the range of countermeasures for each threat and highlight any gaps or issues (ASPR).	According to ASPR officials, senior leaders from across HHS—including ASPR, CDC, FDA, and NIH—conducted portfolio reviews of major investments in the efforts for anthrax, smallpox, radiological/ nuclear, and pandemic influenza threats. After the reviews concluded in March 2011, the leaders developed a list of key challenges within and across the portfolios and assigned responsibility for addressing those challenges to the appropriate agencies and offices.
11	Reexamine the statutory framework for how liability protection is provided.	Review of the Public Readiness and Emergency Preparedness Act and its framework for providing appropriate liability protection for the development, testing, manufacture, and administration of medical countermeasures without commercial uses (ASPR).	According to HHS officials, they have begun to identify challenges with and possible improvements to the Public Readiness and Emergency Preparedness Act.
12	Update the requirements for current and future products.	Review of underlying planning assumptions and requirements in order to develop a more flexible and capabilities-based strategy (ASPR).	According to HHS officials, they plan to develop planning scenarios for high-priority threats and create a prioritized list of requirements. They also plan to determine—and gain PHEMCE approval on—the type of information that should be included in the requirements for certain scenarios and for certain products.
13	Develop multiyear budget planning process.	Coordinated multiyear process to plan for lifecycle costs across PHEMCE (HHS Assistant Secretary for Financial Resources).	HHS officials said that they have begun to develop a 5-year budget planning process to help the department determine how to fund medical countermeasures as they move through the stages of development, acquisition, and stockpile replenishment and as responsibility for the countermeasures moves from one HHS agency or office to another.

Source: GAO analysis of HHS information.

Appendix III: Comments from the Department of Health and Human Services



DEPARTMENT OF HEALTH & HUMAN SERVICES

OFFICE OF THE SECRETARY

Assistant Secretary for Legislation
Washington, DC 20201

SEP 1 2011

Marcia Crosse, Director
Health Care
U.S. Government Accountability Office
441 G Street NW
Washington, DC 20548

Dear Ms. Crosse:

Attached are comments on the U.S. Government Accountability Office's (GAO) draft report entitled: "NATIONAL PREPAREDNESS: Improvements Needed for Acquiring Medical Countermeasures Against Threats from Terrorism and Other Sources" (GAO-11-840).

The Department appreciates the opportunity to review this report before its publication.

Sincerely,

A handwritten signature in cursive script that reads "Jim R. Esquea".

Jim R. Esquea
Assistant Secretary for Legislation

Attachment

**General Comments of the Department of Health and Human Services (HHS) on the
Government Accountability Office's (GAO) Draft Report Entitled: "National
Preparedness: Improvements Needed for Acquiring Medical Countermeasures Against
Threats from Terrorism and Other Sources (GAO-11-840)"**

The Department of Health and Human Services (HHS) appreciates the opportunity to provide comments to the Government Accountability Office's (GAO) report *National Preparedness: Improvements Needed for Acquiring Medical Countermeasures Against Threats from Terrorism and Other Sources*.

Tragic events such as the anthrax attacks of 2001 and the H1N1 influenza pandemic of 2009 underscore the reality that our nation remains vulnerable to deliberate chemical, biological, radiological, and nuclear (CBRN) threats as well as pandemic and other emerging infectious diseases. HHS agrees with the Government Accountability Office (GAO) that efficient interagency coordination is critical to effective development and acquisition of medical countermeasures to counter CBRN threats as well as pandemic and other emerging infectious diseases. In August 2010, the HHS Secretary issued the Public Health Emergency Medical Countermeasures Enterprise Review (2010 MCM Review) that assessed all aspects of medical countermeasure development, from concept to approval. Using these recommendations, HHS has implemented a strategic approach toward strengthening all phases of the medical countermeasure pipeline. HHS launched numerous efforts to strengthen coordination among relevant agencies with the goal of supporting the efficient development and approval of medical countermeasures. Other processes implemented under this strategy ensure HHS is able to more effectively oversee the full life-cycle management of medical products and has a robust operational component to support the nation's capability to respond. A key focus of many agency activities within this strategy has been to reduce uncertainties for developers of promising medical countermeasures and to expand communications among relevant agencies and between the agencies and prospective developers.

The Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) is the overarching interagency convening body that coordinates the multiple efforts and programs that enable the nation to respond to CBRN threats as well as to pandemic and emerging diseases. The Assistant Secretary for Preparedness and Response (ASPR) leads the PHEMCE, which brings together three primary HHS internal agencies: the National Institutes of Health, National Institute of Allergy and Infectious Diseases (NIH/NIAID), the Centers for Disease Control and Prevention (CDC), and the Food and Drug Administration (FDA), along with four key interagency partners: Department of Homeland Security (DHS), Department of Defense (DoD), Department of Veterans Affairs (VA) and Department of Agriculture (USDA). Together, these Enterprise agencies and organizations work to optimize preparedness for public health emergencies in connection with the creation, stockpiling and use of medical countermeasures. The agencies engage directly by regular participation in Enterprise-led workgroups and meetings¹ as well as on numerous scientific and technical teams.² The PHEMCE's efforts are

¹ Examples include the Enterprise Senior Council, the Enterprise Executive Committee, the Integrated Program Teams, and the Project Coordination Teams, among others.

² Examples include the Filovirus Animal Non-Clinical Group and the Inter-agency Diagnostics Working Group.

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already helping to foster information-sharing and education among agencies, medical countermeasure developers and other relevant external stakeholders. The Enterprise is specifically responsible for the following:

- Defining and prioritizing requirements for public health emergency medical countermeasures;
- Focusing research, development and procurement activities on the identified requirements; and
- Establishing deployment and use strategies for medical countermeasures in the Strategic National Stockpile.

Overarching accomplishments by the PHEMCE over the recent past include:

- Developed a strategy to track progress in implementing the major initiatives called for in the 2010 MCM Review.
- In May 2010 and over the course of the last year, instituted the first ever full portfolio reviews for priority threats, including anthrax, smallpox, radiological /nuclear threats and pandemic influenza. These reviews identified gaps across the life cycle of products—from setting requirements through product development and procurement, into stockpile and use. Important gaps were captured as Priority Action Items and are being tracked via a biweekly process to assure they are addressed.
- Instituted a "Case Management" approach for specific products or product areas that require the concerted effort of NIH/NIAID, ASPR/BARDA, CDC, and FDA to solve issues related to the setting of product specific requirements up to advanced development, approval and procurement. Case Management is defined as a new process to increase attention on a specific product or program for the purpose of accelerating solutions for particular advanced development requirements and engaging a dedicated team from across the agencies to effect the needed changes.
- Established a governance structure for the PHEMCE which has effectively overseen all programmatic and policy efforts and includes teams from the program level up to senior leadership. Teams include Integrated Product Teams, a Portfolio Advisory Committee, the Enterprise Executive Committee and the Enterprise Senior Council.
- Quarterly convened the Enterprise Senior Council (ESC) to provide strategic oversight and decision making on key issues, such as a systematic process for identifying the major scenarios against which PHEMCE requirements must be developed. The ESC has also been instrumental in developing consensus advice around major procurement actions under consideration by the ASPR and BARDA.
- Improved outreach and communication with stakeholders and industry partners on current medical countermeasure investment and prioritization including:
 - The ASPR-maintained website www.medicalcountermeasures.gov, which provides up-to-date information on medical countermeasure advanced research and development and procurement contracts, as well as broad policy documents issued by the White House and/or Congress that may impact medical countermeasure programs.
 - Biomedical Advanced Research and Development Authority's (BARDA) Stakeholders Workshop and Industry Day during which participants receive

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information on BARDA's strategy, goals, and areas of interest; its roles and responsibilities, the ASPR Office of Acquisitions Management, Contracts, and Grants (AMCG), and Contractors in contract administration and performance; BARDA's core capabilities to support product development; new initiatives designed to address the challenges associated with developing medical countermeasures; and opportunities to develop medical countermeasures for both government and commercial markets. In addition, at the request of participants, BARDA program staff and/or AMCG Contracting Officers are available to meet one-on-one.

Agency Contributions to PHEMCE Efforts:

BARDA is coordinating an integrated, systematic approach to the development and purchase of necessary vaccines, drugs, therapies, and diagnostic tools for use during a public health emergency. It manages Project BioShield, which pursues the procurement and advanced development of medical countermeasures for CBRN threats. BARDA also works to develop and procure medical countermeasures for pandemic influenza and other emerging infectious diseases that fall outside the auspices of Project BioShield. In addition, over the past year, BARDA has implemented routine in-process reviews (IPRs) to evaluate progress, obstacles, challenges, and solutions for medical countermeasure development and manufacturing projects facing pivotal milestones. IPRs, which are conducted in consultation with NIAID, CDC, DoD, and FDA, provide useful technical and regulatory evaluation and advice to contractors outside of the usual framework between FDA and product sponsors. Private sector partners have expressed their appreciation for this new forum for discussion of product development and regulatory issues with all members of the Enterprise. The IPRs have provided FDA with an opportunity to interface with product sponsors much earlier in the development process for very specific indications against CBRN, pandemic influenza and other emerging infectious disease threats. BARDA has furthermore instituted a Case Management approach to assist contractors needing support in technical, clinical, and regulatory areas. BARDA partners with CDC, NIAID and FDA staff and senior leaders on a regular basis to address specific challenges and issues in order to move a candidate product toward approval and/or acquisition.

The FDA regulates all medical products within the PHEMCE and is integral to strategic success in approving products for procurement or assessing emergency use approvals for those awaiting final approval. In response to the 2010 MCM Review, FDA launched an MCM initiative to build upon ongoing medical countermeasure activities and expand communication and collaboration with federal partners and prospective developers. The initiative aims to overcome regulatory hurdles, reduce regulatory uncertainties, and anticipate and communicate to developers the new regulatory strategies needed to develop novel concepts and obtain approval of candidate products. FDA is working closely with BARDA, CDC, DoD and NIH in the earliest stages of requirements setting and product development to make sure that appropriate regulatory requirements are clear and that product developers are able to gather the necessary data for determining safety and efficacy. FDA is establishing internal collaborations and Action Teams to identify regulatory holdups and recommend changes to improve product review processes. Regulatory science research is already under way to develop innovative tools to support novel medical countermeasure development.

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The CDC develops and exercises a concept of operations to deliver and dispense medical countermeasures where and when needed. CDC is the critical link to state, local, territorial and tribal end-users, thus representing within the PHEMCE the voice of the public health and medical community needs in product development and deployment. CDC also manages the Strategic National Stockpile and addresses the logistical requirements and allocation of resources for maintaining and replacing its multiple products.

NIAID oversees a robust and forward-looking research program that informs advanced development efforts through exploring scientific knowledge of threats and producing a pipeline of candidate medical countermeasures. NIAID has established the Concept Acceleration Program to accelerate this transition as described in the 2010 MCM Review. This effort will actively identify promising technologies and concepts and will provide added funding and access to services to more effectively incubate and transition good concepts into advanced development potential.

PHEMCE efforts are already underway to ensure the continued success of these important programs. Component organizations have developed five-year spending plans and have identified strategic goals that reflect the priorities of the enterprise. HHS concurs with GAO that the 2007 PHEMCE Strategy and Implementation Plans for Chemical, Biological, Radiological and Nuclear Threats should be updated and, as noted in the report, efforts have already begun to enable the release of such updated Plans in Spring 2012. A Steering Committee to develop these Plans for ultimate review and approval by the Enterprise Senior Council has been established and will be led by ASPR staff. The Steering Committee has begun discussions concerning, among other critical issues, how best to frame and communicate anticipated spending levels for the highest priority medical countermeasure programs. Any such estimates must consider the future status of the Special Reserve Fund which funds Project BioShield contracts, as well as the annual appropriations process that funds the vast majority of PHEMCE activities. At this time, the information and subsequent prioritization included in the 2007 PHEMCE is still relevant. Priorities identified in the 2007 PHEMCE Implementation plan were based on DHS MTDs and other available information. Since 2007, necessary countermeasures for CBRN threats have not significantly changed.

The high-level snapshots of activities underway reflect the significant and ongoing investment of energy and resources for medical countermeasure research, development, acquisition, procurement, and distribution to better prepare the nation against CBRN, pandemic and other emerging infectious disease threats. These investments also demonstrate the federal public health community's long-term commitment to this critical national effort. HHS is committed to ensuring the nation is prepared for a potential CBRN event and pandemic or emerging infectious disease threat and will continue to refine its medical countermeasure priorities and investments.

Appendix IV: GAO Contact and Staff Acknowledgments

GAO Contact

Marcia Crosse, (202) 512-7114 or crossem@gao.gov

Acknowledgments

In addition to the contacts named above, Sheila K. Avruch, Assistant Director; Shana R. Deitch; Tracey King; Corissa Kiyon; Carolina Morgan; Roseanne Price; and Jessica C. Smith made significant contributions to this report.

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