DEPARTMENT OF HEALTH & HUMAN SERVICES Public Health Service

Food and Drug Administration
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"HHS Agencies' Efforts to Prepare the Nation to Combat Biological Events"

Statement of
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Introduction

Good morning Chairman Kingston, Ranking Member DeLauro, and members of the Subcommittee. I am Dr. Luciana Borio, Assistant Commissioner for Counterterrorism Policy, Director of the Office of Counterterrorism and Emerging Threats, and Acting Deputy Chief Scientist at the Food and Drug Administration (FDA). Thank you for the opportunity to appear today to discuss FDA's efforts to prepare our nation to mitigate biological threats – such as a biological weapons attack or naturally emerging infectious diseases like pandemic influenza and antimicrobial resistant pathogens.

FDA's Medical Countermeasure Mission

FDA plays a critical role in protecting the United States from deliberate chemical, biological, radiological and nuclear (CBRN) threats and naturally occurring infectious diseases. Specifically, FDA is responsible for ensuring that medical countermeasures—including drugs, vaccines, and diagnostic tests—to counter these threats are safe, effective, and secure. The mission of my office is to facilitate the development and availability of these life-saving products.

Collaboration is the cornerstone of this critical public health responsibility. FDA works closely with its interagency partners through the Department of Health and Human Services (HHS) Public Health Emergency Medical Countermeasures Enterprise (Enterprise) to build and sustain the medical countermeasure programs necessary to respond effectively to public health emergencies. FDA also works closely with the U.S. Department of Defense (DoD) to facilitate the development and availability of medical countermeasures to support the unique needs of the warfighter. For example, FDA supports the Enterprise and DoD by providing subject matter expertise for developing medical countermeasures. We also provide scientific and regulatory counsel to inform medical countermeasure stockpiling and deployment decisions. In addition, FDA employs its authorities, such as Emergency Use Authorization (EUA), to facilitate access to medical countermeasures to respond to public health and military emergencies, even when products are not yet approved for any use or the particular use needed.

In 2010, FDA launched its Medical Countermeasures initiative (MCMi), focusing increased resources on identifying and resolving regulatory challenges to medical countermeasure development and availability. Within FDA, MCMi promotes the development of medical countermeasures by establishing clear regulatory pathways for medical countermeasures, instituting effective regulatory policies and mechanisms to facilitate timely access to available medical

countermeasures, and advancing medical countermeasure regulatory science to create the tools that support regulatory decision-making.

Launching MCMi at FDA

Just prior to the close of FY 2010, FDA received $170 million in one-time, no-fiscal year funding to immediately commence MCMi activities. FDA used these resources to establish the MCMi program, hire 77 FTEs and conduct regulatory science activities to address and resolve important questions related to medical countermeasures. Between FY 2011 and FY 2013, FDA expended $126.3 million of its no-year funding for this purpose, and we expect to spend the remaining $12.2 million on related research during FY 2014 and FY 2015.

Between FY 2012 and FY 2014, FDA received core funding from Congress to sustain and modestly expand its MCMi program. For FY 2012, FDA received an appropriation of $20.0 million to support MCMi activities that we funded in previous fiscal years with one-time money. The FY 2012 appropriation allowed FDA to sustain 70 of its 77 MCMi FTEs and support a $327,000 investment in MCM regulatory science. In FY 2013, Congress increased FDA's budget for MCMi by $3.5 million, providing a total MCMi base of $23.5 million. The sequestration and rescission diminished some of the MCMi FY 2013 budget increase. However, the FY 2013 funding level allowed FDA to support the 77 FTE initially hired with no-year funds as well as increase our annual investment in MCM regulatory science to $1.4 million. In FY 2014, Congress provided FDA with a $1.0 million increase for MCMi, for a total base of $24.5 million. FDA will primarily use this increase to support FTE costs.

Scope of MCMi Operations

FDA's scope of operations within the medical countermeasures responsibilities covers a broad range of activities essential to facilitating development and access to safe and effective medical countermeasures, including:

- Reviewing medical countermeasure marketing applications and approving those that meet applicable standards for safety and efficacy;
- Providing regulatory advice, guidance and – when needed – technical assistance to medical countermeasures product sponsors and our Enterprise partners;
- Supporting efforts to establish and sustain an adequate supply of medical countermeasures, including averting supply disruptions when feasible and, in certain situations, allowing products to be used beyond their expiration dates when supported by our scientific analyses;

- Supporting the development of advanced manufacturing technologies by collaborating with the HHS Biomedical Advanced Research and Development Authority (BARDA) on their Centers for Innovation in Advanced Development and Manufacturing;
- When necessary, enabling access to medical countermeasures that are not yet approved for use through an appropriate mechanism, such as EUA;
- Ensuring that FDA regulations and policies adequately support medical countermeasures development and enable preparedness and response activities;
- Fostering the professional development of our scientists to ensure that FDA personnel maintain the skills and abilities to support the medical countermeasure mission;
- Proactively identifying and resolving regulatory challenges associated with medical countermeasures; and
- Improving medical countermeasure development timelines and success rates through an MCMi Regulatory Science Program that harnesses cutting-edge science and applies innovative approaches to answer questions that are vital to FDA regulatory decisions.

Measures of Success

The additional resources that Congress provided to FDA for the MCMi have enabled FDA to hire expert staff and become more deeply and thoroughly engaged in medical countermeasure activities throughout the Enterprise. With this increased engagement, FDA has helped to resolve many regulatory challenges and impediments associated with the U.S. government's advanced development medical countermeasure pipeline so that medical countermeasure development does not stall and continues to move forward. For example, this has resulted in the recent approval, licensure, or clearance of several medical countermeasures, including an inhalational anthrax therapeutic, a botulism antitoxin, a next-generation portable ventilator, and several influenza diagnostic tests. Of note, FDA was able to approve the inhalational anthrax therapeutic and the botulism antitoxin for use in children as well as adults – despite the fact that pediatric patients were not studied due to ethical concerns during the development of these products. These achievements were made possible by the application of regulatory science. In addition, FDA also expanded approval for use of the influenza antiviral, oseltamivir, to treat children as young as 2 weeks of age. Prior to this action, oseltamivir was only approved to treat influenza in children ages 1 year and older. FDA was able to expand the approved use of oseltamivir in children younger than 1 year based on the extrapolation of data from previous studies of adults and older children, and additional supporting studies sponsored by both industry and academic researchers.

FDA has also readied medical countermeasures for potential use under its EUA authorities against a diverse array of threats including smallpox, anthrax, and pandemic influenza.[1] Furthermore, FDA issued EUAs for diagnostic tests for the avian influenza A (H7N9) virus and Middle East Respiratory Syndrome coronavirus (MERS-CoV) to facilitate preparedness for these emerging biological threats.

In the area of pandemic influenza preparedness, FDA recently approved several seasonal influenza vaccines, which helps increase and sustain pandemic influenza vaccine production capacity, including: the first seasonal influenza vaccine licensed in the U.S. produced using modern cell culture techniques, which helps to ensure a faster manufacturing startup; and the first seasonal influenza vaccine made through recombinant DNA technology, which speeds vaccine production. FDA also approved the first adjuvanted influenza vaccine for use in people 18 years of age and older who are at increased risk of exposure to the avian influenza H5N1 virus subtype contained in the vaccine. This vaccine is not for commercial distribution, but will be part of the national stockpile in the event it is needed. Furthermore, FDA has collaborated closely with BARDA, the National Institute of Allergy and Infectious Diseases (NIAID), and Centers for Disease Control and Prevention (CDC) on developing avian influenza H7N9 virus vaccine candidates.

FDA has also established a broad and robust portfolio of cutting-edge research under MCMi's Regulatory Science Program. A few examples of ongoing projects include: supporting the Wyss Institute for Biologically Inspired Engineering at Harvard University as it develops organs-on-chips models to assess radiation damage in lung, gut, and bone marrow, and then using these models to test candidate medical countermeasures; assessing the feasibility of using electronic health record systems to conduct near real-time monitoring of health outcomes, including clinical benefit or serious or unexpected adverse events associated with medical countermeasures used during public health emergencies; collaborating with the Defense Threat Reduction Agency and the National Center for Biotechnology Information to establish a publicly-available genomic sequence reference database that will be critical to developers seeking to validate their candidate multiplex *in vitro* diagnostic tests that could be used to diagnose multiple pathogens simultaneously; and

[1] To facilitate the issuance of EUAs, FDA has developed a pre-EUA submission process. FDA works with product sponsors or government agencies, such as CDC and DoD, to develop pre-EUA packages that will form the basis of an EUA request and decision when circumstances justify. Pre-EUA packages contain data and information about the safety and efficacy of the product, its intended use under an EUA, and information about the potential emergency situation that might unfold.

examining the scientific basis for the instability of the protective antigen that have hindered efforts to develop next-generation anthrax vaccines and using protein engineering to stabilize the antigen.

Details of these and other FDA successes across the full range of FDA activities within the medical countermeasure mission space appear in our annual MCMi program updates.[2]

Coordination with the Enterprise

FDA coordinates and collaborates extensively with Enterprise partners to foster the development and availability of medical countermeasures. FDA provides subject matter expertise and technical assistance to numerous standing Enterprise committees and working groups that develop medical countermeasure requirements, plans, priorities, and policies and that conduct program oversight and integration across the full spectrum of activities associated with medical countermeasures: threat assessments, requirements setting, product development, and procurement, stockpiling, and use.

As FDA implements its MCMi, we also engage with our Enterprise partners to ensure programmatic alignment and the most efficient use of resources. For example, FDA established a Steering Committee for Advancing MCMi Regulatory Science. This committee—which includes representatives from NIAID, the CDC, BARDA, and DoD—evaluates MCMi Regulatory Science Program research proposals for feasibility and scientific and technical merit to ensure that the MCMi Regulatory Science Program is appropriately targeted and coordinated with U.S. government medical countermeasure priorities.

Conclusion

Developing medical countermeasure is highly complex. Close cooperation and collaboration, within FDA and with Enterprise partners, is essential, and without this cooperation, the progress to address this public health challenge would be very limited. The deep engagement that is evident among the agencies represented here today is an example of public health synergy at its best. FDA is fully committed to continuing to work closely with Enterprise partners and product developers to support the development of promising medical countermeasures and to facilitating their ready availability should they be needed to respond to a public health emergency.

Thank you and I am happy to answer your questions.

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[2] FDA's Annual MCMi Program Updates are available at http://www.fda.gov/EmergencyPreparedness/MedicalCountermeasures/ucm270744.htm