DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

Food and Drug Administration
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STATEMENT

OF

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BEFORE THE

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COMMITTEE ON HEALTH, EDUCATION, LABOR AND PENSIONS

"MEDICAL AND PUBLIC HEALTH PREPAREDNESS AND RESPONSE: ARE WE READY FOR FUTURE THREATS?"

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INTRODUCTION

Good morning Mr. Chairman, Ranking Member, and Members of the Committee. 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 or the Agency). Thank you for the opportunity to appear today to discuss FDA's efforts to prepare our nation to mitigate chemical, biological, radiological, and nuclear (CBRN) threats as well as threats from naturally emerging infectious diseases like pandemic influenza and antimicrobial-resistant pathogens.

FDA's Medical Countermeasures Mission

FDA plays a critical role in facilitating the development and availability of medical countermeasures to protect the United States from CBRN and emerging infectious disease threats. FDA works closely with its interagency partners through the Public Health Emergency Medical Countermeasures Enterprise (the Enterprise) to build and sustain the medical countermeasure programs necessary to respond effectively to public health emergencies. We also work with the U.S. Department of Defense (DoD) to facilitate the development and availability of medical countermeasures to support the unique needs of the military. The mission of my office is to facilitate the development and availability of these lifesaving products.

In 2010, FDA launched its Medical Countermeasures Initiative (MCMi) to focus increased resources on facilitating the development and availability of medical countermeasures. FDA's scope of operations within its medical countermeasures mission covers a broad range of activities

vital to facilitating the development of 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 appropriate and needed—technical assistance to medical countermeasures product sponsors, U.S. Government partners, international regulators, and international organizations such as the World Health Organization (WHO);
- 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 Department of Health and Human Services (HHS) Biomedical Advanced Research and Development Authority (BARDA) on their Centers for Innovation in Advanced Development and Manufacturing;
- Facilitating access to medical countermeasures that are not yet approved, licensed, or cleared for use, if circumstances warrant, through an appropriate mechanism such as an Emergency Use Authorization (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 countermeasures mission;
- Proactively identifying and resolving regulatory challenges associated with medical countermeasures; and

- Supporting regulatory science to develop the tools, standards, and approaches necessary to assess the safety and efficacy, including quality and performance, of medical countermeasures.

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. This increased engagement, along with new authorities gained under the Pandemic and All-Hazards Preparedness Reauthorization Act (PAHPRA) of 2013, which, for example, provided FDA greater flexibility in the issuance of EUAs, has enabled FDA to better respond to emerging public health threats. For example, FDA worked proactively with U.S. Government partners, international partners, and product developers to help facilitate the development and availability of medical countermeasures to respond to the avian influenza A (H7N9) virus and the Middle East Respiratory Syndrome coronavirus (MERS-CoV). FDA authorized the use of three diagnostic tests for the H7N9 virus and one diagnostic test for MERS-CoV, under its EUA authority.

FDA has also taken extraordinary steps to be proactive, flexible, and highly responsive to the Ebola epidemic in West Africa, which has presented a complex range of issues. FDA's efforts include:

- Working to help expedite the development and availability of medical products to detect, prevent, and treat Ebola virus disease, by providing scientific and regulatory advice to commercial developers and U.S. Government agencies that support medical product development;

- Working with product sponsors, international regulators, WHO, and the National Institutes of Health (NIH) to launch Ebola vaccine trials in record time;
- Collaborating with NIH to help with the design of an innovative and robust common clinical trial protocol to evaluate investigational treatments for Ebola;
- Collaborating extensively with WHO and our international regulatory counterparts to exchange information about investigational products for Ebola in support of international response efforts and to achieve regulatory harmonization, where possible;
- Facilitating access to investigational medical products for patients with Ebola, when requested by clinicians;
- Authorizing the use of seven diagnostic tests for Ebola under our EUA authority; and
- Actively monitoring for fraudulent products that claim to diagnose, prevent, or treat Ebola infection and taking action, as warranted, to protect public health (for example, FDA issued Warning Letters to six firms marketing products that claim to prevent, treat, or cure infection with the Ebola virus).

FDA's increased engagement under the MCMi has also helped to resolve many challenges and impediments associated with the U.S. Government's medical countermeasures pipeline so that development programs continue to move forward. For example, this has resulted in the approval of several medical countermeasures, including a therapeutic for inhalational anthrax, a botulism antitoxin, two antibiotics for the treatment and prophylaxis of plague, and a next-generation portable ventilator. Of note, FDA was able to approve the anthrax therapeutic 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 this product. This achievement was made possible by the application of regulatory science.

FDA has also continued its efforts to support the establishment and sustainment of an adequate supply of medical countermeasures. For example, FDA supports the Shelf Life Extension Program (SLEP), a Federal fee-for-service program, for extending the useful shelf life of military-significant and contingency-use medical products, including medical countermeasures that are owned by components of DoD or other Federal program participants, such as the Strategic National Stockpile (SNS).[1] FDA laboratory personnel test and evaluate drugs submitted for shelf-life extension to ensure stability and quality before a shelf-life extension is approved.

In addition, FDA has continued to work to ensure that the U.S. Government is as prepared as possible to rapidly deploy medical countermeasures when necessary. For example, FDA has readied stockpiled medical countermeasures for potential use under its EUA authorities against a diverse array of threats including smallpox, anthrax, and pandemic influenza.[2]

In the area of pandemic influenza preparedness, FDA has approved several influenza diagnostic tests, which can help facilitate an effective response to an influenza pandemic by rapidly identifying infected persons and facilitating appropriate containment measures and clinical care. In addition, FDA has approved several seasonal influenza vaccines, which helps increase and sustain pandemic influenza vaccine production capacity, including the first seasonal influenza vaccine licensed in the United States, produced using modern cell culture techniques, and the first seasonal influenza vaccine made through recombinant deoxyribonucleic acid (DNA) technology. Both of these

[1] SLEP is designed to defer drug replacement costs for date-sensitive stockpiles of drugs by extending their useful shelf life beyond the manufacturer's original expiration date.

[2] 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.

vaccines offer an alternative to the egg-based process and a potential for a faster manufacturing startup in the event of a pandemic. 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 CDC on developing avian influenza H7N9 virus vaccine candidates.

Additionally, FDA has approved the first intravenous antiviral drug to treat acute, uncomplicated influenza infection in adults and has expanded approval for use of an influenza antiviral, oseltamivir, to treat children as young as two weeks of age. Prior to this action, oseltamivir was only approved to treat influenza in children ages one year and older. FDA was able to expand the approved use of oseltamivir in children younger than one year old 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.

Consistent with the President's September 18, 2014, Executive Order and the National Strategy for Combating Antibiotic Resistant Bacteria, FDA is working hard to help ensure the development of new products in preparation for threats from naturally emerging infectious diseases from antimicrobial-resistant pathogens. Toward this end, FDA will evaluate new antibacterial drugs for patient treatments, streamline clinical trials, help phase out the use of medically important antimicrobials in food-producing animals, develop better vaccines for antibiotic resistant organisms, and strengthen capacities to detect antibiotic resistance.

On the regulatory science front, FDA has established a broad and robust portfolio of cutting-edge research under MCMi's Regulatory Science Program to help develop the tools necessary to support regulatory decision-making. A few examples of ongoing projects include supporting the Wyss Institute for Biologically Inspired Engineering at Harvard University as it develops models to assess radiation damage in lung, gut, and bone marrow, and then using these models to test candidate medical countermeasures; collaborating with DoD and the National Center for Biotechnology Information to establish a publicly available, well-curated, high-quality, whole microbial genome sequence reference database from clinically important pathogens, which diagnostic test manufacturers will be able to use to advance their sequence-based test development programs; and examining the scientific basis for the instability of the protective antigens that has hindered efforts to develop next-generation anthrax vaccines and using protein engineering to stabilize the antigen.

CONCLUSION

Developing and enabling ready access to medical countermeasures to mitigate CBRN and emerging infectious diseases is highly complex. Close cooperation and collaboration within FDA and with U.S Government, international, and private sector partners are essential, and without this cooperation and collaboration, progress to address this growing 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 our partners, using our authorities to the fullest extent possible to protect and promote public health, both domestically and abroad, in response to public health threats.

Thank you and I am happy to answer your questions.