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STATEMENT

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BEFORE THE

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"Examining Medical Product Development in the Wake of the Ebola Epidemic"

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INTRODUCTION

Good morning Chairman Pitts, Ranking Member Pallone, 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 or the Agency). Thank you for the opportunity to appear today to discuss FDA's response to the Ebola epidemic in West Africa—the worst in recorded history.

The toll of this epidemic, with so many lives lost and so many others fighting for their lives, is heartbreaking and tragic. While the outbreaks in Senegal and Nigeria were rapidly contained by the application of standard public health techniques and have now been declared over, disease transmission continues in Guinea, Liberia, and Sierra Leone. In addition, a small cluster of Ebola cases has recently been reported in Mali.

The primary approach to containing the epidemic remains standard public health measures, such as identifying and isolating infected individuals, caring for patients who are ill, ensuring that health care workers have access to personal protective equipment and are properly trained in infection control measures, and tracing patients' contacts to detect any secondary infections, as soon as possible. However, applying these public health measures on a large scale presents complex challenges because of the strains on health care and public health infrastructure within affected countries and the very limited capacity to provide supportive medical care in-country. This tragic situation is further complicated because there are no treatments or vaccines shown to be safe or effective for treating or preventing Ebola virus disease, and products currently under

development are in the very early stages of investigation for their respective indications. FDA is dedicated to do all that we can to respond effectively and rapidly to this epidemic.

FDA's Response to the Ebola Epidemic

This Ebola outbreak is an unprecedented global health and security crisis, and FDA is taking extraordinary steps to be proactive and flexible in our response. We have a critical role in helping to facilitate the development, manufacturing, and availability of investigational products for use against Ebola virus disease. In response to this urgent situation, FDA is actively working with Federal colleagues, industry, and international organizations to facilitate development, including the evaluation of safety and efficacy, of treatments and vaccines with the potential to help mitigate this epidemic.

Each Federal partner has a vital part to play in the global race to find therapeutic solutions to this deadly disease. FDA participates in a cross-cutting Federal workgroup that meets regularly to provide ongoing interactions between the different Federal participants. FDA provides scientific and regulatory advice to U.S. Government agencies that support medical product development, including the National Institute of Allergy and Infectious Diseases (NIAID) at the National Institutes of Health (NIH), the Biomedical Advanced Research and Development Authority (BARDA) in the Office of the Assistant Secretary for Preparedness and Response (ASPR), and the U.S. Department of Defense (DoD), to help speed their development programs. We are also closely coordinating our activities with the Centers for Disease Control and Prevention (CDC).

In addition, we are extremely engaged with multiple medical product developers to clarify regulatory requirements, provide input on pre-clinical and clinical trial designs (including the potential for clinical trials that could use a common protocol to test several products at once and

that can be conducted in affected countries and in the United States), and expedite review of data as they are received from product developers. As a result, it is generally recognized that investigational products, including vaccines, for Ebola are moving forward at an unprecedented pace. As part of the overall response, FDA is expediting the review of Ebola-related Investigational New Drug (IND) applications, which are required by law for FDA-regulated clinical trials of drugs and vaccines to proceed. For example, FDA reviewed IND applications for investigational Ebola vaccines in less than one week and, after such review, allowed them to proceed. NIAID, which is co-developing an Ebola vaccine with GlaxoSmithKline (GSK), has announced that it began Phase 1 clinical testing in early September of this year, and NewLink Genetics has announced beginning Phase 1 clinical trials of its Ebola vaccine candidate in October. We also continue to work closely with therapeutic product developers to speed development of their products. To augment diagnostic capacity, we have contacted several commercial developers—entities we know are capable of rapidly developing these types of diagnostic tests—and have encouraged them to work with us to quickly develop and make available such tests. Several entities have expressed interest and are now in discussions with FDA.

FDA also is collaborating with the World Health Organization (WHO) and working with several of our international regulatory counterparts, including the European Medicines Agency, Health Canada, and others, to exchange information about investigational products for Ebola. These efforts support regulatory collaboration to harmonize and accelerate development and have the potential to contribute to approval of medical products in the United States and in other nations. With this important goal in mind, FDA entered into a confidentiality commitment with WHO to allow the exchange of non-public information concerning medical products. We believe this will

facilitate international collaboration to respond to the Ebola epidemic, as well as more broadly to prepare for and respond to any future public health crises.

I have had the opportunity to participate in several WHO-sponsored consultations with my Federal colleagues, as well as representatives of the international public health community and medical product sponsors, to discuss the leading investigational treatments and vaccines for Ebola and key considerations for deployment in West Africa. These consultations are fostering a more coordinated and effective global public health response to the Ebola epidemic. Additionally, FDA scientists are providing technical advice to the WHO as they work to assess the role of convalescent plasma in ameliorating Ebola virus disease. Moving forward, FDA will continue working with our international colleagues to foster development of and access to investigational products in affected countries.

While FDA is making every effort to encourage development, speed review, and use flexible approaches to enhance the availability of potential medical products to address Ebola, it must be remembered that the investigational vaccines and treatments for Ebola are in the earliest stages of development. Data on effectiveness in humans are limited or lacking, and accurate assessment may be impossible if adequately designed clinical trials are not performed. In addition, the supply of some investigational products is limited. This supply issue constrains the options for properly assessing the safety and efficacy of these investigational products in clinical trials to respond to the epidemic, and also limits the possibilities for making products available for therapeutic use outside of a clinical trial (also known as expanded access). FDA is working with NIH to develop a flexible, innovative and adaptive clinical trial protocol that will provide a mechanism for product sponsors and investigators to evaluate multiple investigational products

under a common protocol. Our shared goal is to ensure accrual of interpretable data and generate actionable results in the most expeditious manner.

While investigational products are being developed, with the ultimate goal of approval of safe and effective products and manufacturing for wide-scale use, FDA is doing all it can to facilitate access to these products when access has been granted by the sponsor and the clinical circumstances warrant. FDA has one of the most flexible regulatory frameworks in the world, which includes mechanisms to enable access to investigational medical products when appropriate, after the risks and benefits to the patient have been weighed.

In addition, under the FDA's Emergency Use Authorization (EUA)[1] authority, we can allow the use of an unapproved medical product—or an unapproved use of an approved medical product—for a larger population during certain emergencies, when, among other reasons, sufficient preliminary data on potential risks and benefits are available for review and there is no adequate, approved, and available alternative. To date, FDA has authorized the use of six Ebola diagnostic tests (one developed by DoD, two developed by CDC, and three sponsored by commercial manufacturers). These diagnostic tests can help facilitate an effective response to the epidemic by rapidly identifying patients infected with Ebola virus and facilitating appropriate containment measures and clinical care. We were able to issue these EUAs, in part, because of new authorities gained under the Pandemic and All-Hazards Preparedness Reauthorization Act of 2013, or PAHPRA, which provide greater flexibility in the issuance of EUAs.

[1] Under the FD&C Act, as amended by the Project BioShield Act of 2004 [PL 108-276] and PAHPRA [PL 113-5], the Secretary of HHS has the authority to authorize FDA to permit the "emergency use" of medical countermeasures in certain situations [21 USC § 360bbb-3].

Unfortunately, during epidemics such as this, fraudulent products that claim to prevent, treat, or cure a disease rapidly appear on the market. FDA has learned of several fraudulent products that claim to prevent or treat Ebola virus infection. In response, we issued a statement, warning consumers about fraudulent Ebola treatment products, and we are taking actions against fraudulent claims to protect public health. For example, we issued Warning Letters to three firms marketing products that claim to prevent, treat, or cure infection by the Ebola virus.

EMERGENCY APPROPRIATIONS REQUEST

The Administration has requested an additional $6.18 billion to support the U.S. Government's international and domestic efforts to respond to the Ebola epidemic as well as efforts to develop medical products with the potential to help mitigate this epidemic. The request includes $25 million for FDA. This funding is necessary to enable FDA to sustain its aggressive efforts to support the pipeline of investigational medical products for Ebola and other serious emerging infectious diseases, including accelerating product development and review, facilitating access to investigational products, and supporting fraudulent product surveillance. This funding also will enable FDA to support and conduct the regulatory science research necessary to develop the tools, standards, and approaches to characterize investigational medical product safety, efficacy, quality, and performance that are so critical to expedite availability of medical products for Ebola.

CONCLUSION

FDA is fully committed to responding in the most proactive, thoughtful, and flexible manner. We have explored multiple ways to be highly responsive and adaptive to the complex range of

issues that this constantly changing epidemic has presented and will continue to present. FDA is using its authorities to the fullest extent possible to continue its mission to protect and promote the public health, both domestically and abroad.

Developing the medical products to help bring this Ebola epidemic under control is highly complex and will, unfortunately, take time. Close cooperation and collaboration within FDA, within the U.S. Government, with our international partners, and with product developers are essential to the global response to this epidemic.

Determining, as quickly as possible, whether the investigational drugs and vaccines being developed for the treatment and prevention of Ebola virus disease work have little effect, or actually harm patients is critically important. The only way to make that determination is through properly designed clinical trials.

FDA is fully committed to sustaining our deep engagement and aggressive response activities. We will continue to work closely with our U.S. Government and international partners and with product developers to speed the development and availability of promising medical products that offer the potential to help end this epidemic as quickly as possible. We fully appreciate the gravity of the situation at hand and are exercising maximum flexibility in our activities. We are singularly focused on facilitating and expediting the development of safe and effective medical products to diagnose, prevent, and treat Ebola virus disease.

Thank you, and I am happy to answer your questions.