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BARDA'S Role in the PHEMCE

Statement of

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Good morning. Chairman Kingston, Ranking Member De Lauro and other distinguished Members of the Subcommittee, thank you for the opportunity to speak with you today about government biodefense efforts. I am Dr. Robin Robinson, Director of the Biomedical Advanced Research and Development Authority (BARDA) and Deputy Assistant Secretary for Preparedness and Response (ASPR) at the Department of Health and Human Services.

Within the Public Health Emergency Medical Countermeasure Enterprise (PHEMCE), BARDA, like its parent organization, ASPR, was established by the Pandemic and All-Hazards Preparedness Act of 2006. Aligned with the PHEMCE strategy and implementation plans, BARDA is the government agency mandated to support advanced development and acquisition of novel and innovative medical countermeasures (MCM) such as vaccines, antimicrobial drugs, diagnostics, and medical devices for the entire nation to address the medical consequences of man-made threats and Mother Nature like the H1N1 pandemic.

Medical countermeasure development is risky, lengthy, and costly with many inexperienced developers failing and larger pharmaceutical companies avoiding the sector completely. BARDA, as a key partner in the PHEMCE, serves as a bridge over a critical gap referred to as the “Valley of Death” in MCM development through direct support, public-private partnerships, and technical core service assistance. Once the PHEMCE establishes product requirements NIH launches discovery and early stage development of product candidates from industry partners then transitioning to BARDA for advanced research and development support and assistance to reach sufficient maturity for product acquisition under Project BioShield, towards FDA approval and

eventual stockpiling at CDC's Strategic National Stockpile (SNS) or the commercial sector. Upon FDA approval, the financial responsibility of MCMs transfers from BARDA under Project BioShield to the SNS to stockpile and deliver. Finally, in public health emergencies BARDA takes a response role by interfacing with other PHEMCE partners and manufacturers to develop, produce, and test products for FDA review and approval and CDC distribution to state and local providers, as illustrated in the 2009 H1N1 pandemic and the H7N9 outbreaks.

BARDA has delivered on its founding mandate to develop products towards regulatory approval and acquire them for national security and public health preparedness. Towards our primary strategic goal, we have become the bridge across the "Valley of Death" a gap in the pharmaceutical development pipeline that obstructs early development candidates from reaching commercial manufacturing capability and ultimate FDA approval. A lack of a commercial market and significant up front commercial investments prevent MCM developers from succeeding. BARDA support and funding facilitate successful product development and have established a robust and formidable product development pipeline of 150+ product candidates. BARDA has procured 12 novel products under Project BioShield since its inception and include smallpox vaccines and antiviral drugs, anthrax vaccines and antitoxins, botulinum antitoxins, radionuclide chelators, anti-neutropenia cytokines for radiation illness, and chemical agent anti-convulsive drugs. BARDA has built a national stockpile of H5N1 and H7N9 vaccines and new adjuvants for pandemic preparedness. Recently, FDA has approved 7 first-in-class products supported by BARDA.

BARDA has created public-private partnerships with industry partners to expand domestic pandemic influenza vaccine manufacturing surge capacity several fold by retrofitting or building new manufacturing facilities in the U.S. BARDA used Other Transactions Authority in 2013 to establish a unique partnership with industry to develop new classes of antimicrobial drugs for biothreats and prominent antimicrobial resistant pathogens such CRE and MRSA – part of BARDA's role in the national war on antimicrobial drug resistance.

Further BARDA has created Core Service Assistance programs to help inexperienced drug developers and provide MCM response capabilities. In 2010, BARDA enlisted 17 laboratories in the U.S. and the U.K. to develop qualified animal models and perform animal challenge studies on CBRN MCMs. In 2012, BARDA established 3 domestic Centers for Innovation in Advanced Development and Manufacturing (CIADM) to assist CBRN product developers routinely with the development and manufacture of products for clinical studies and stockpiling. In an influenza pandemic, these Centers will double as flexible manufacturing facilities rapidly producing millions of vaccine doses. Recently, BARDA established a Fill Finish Manufacturing Network comprised of 4 domestic manufacturers who can aseptically fill pandemic influenza vaccines, assist the Centers with filling needs, and address drug shortages as appropriate. This year, BARDA plans to unveil a Clinical Studies Network comprised of multiple Clinical Research Organizations to address clinical needs of BARDA's product developers and support BARDA's response needs. Together these Core Service Assistance programs effectively mitigate the risk of drug development and make MCMs available in emergencies.

Lastly, BARDA has increased the sustainability of biodefense preparedness and readiness by changing our approach from a “one bug – one drug” paradigm to supporting development of existing drugs that may be repurposed such as anti-neutropenia drugs for radiation treatment and new drugs that have multiple indications including biodefense and commercial public health usages such as new antibiotics for biothreats and community- and hospital-acquired pathogens, especially CRE and MRSA.

Another new tool that the PHEMCE is using to streamline transition of product candidates to one another and address life-cycle management costs is multiyear planning as mandated in the Pandemic and All-Hazards Reauthorization Act. This tool tracks both financial resources and implementation of the PHEMCE Strategic Implementation Plan across PHEMCE partners. BARDA forecasts its product priorities and transitions to PHEMCE partners.

BARDA expects over the next 5 years to receive new product candidates for its advanced development pipeline that will treat viral hemorrhagic fever, multidrug resistant pathogens, and radiation illnesses and provide more effective influenza vaccines. BARDA expects 12 more new MCMs to mature sufficiently in advanced development for acquisition under Project BioShield; these MCMs include next generation anthrax vaccines, better smallpox vaccines, biodosimetry diagnostic devices, thermal burn radiation drug and skin replacement therapies, radiation cell therapies, new antibiotics, and new chemical antidotes, provided appropriations are available.

Lastly, several MCMs that have been approved recently or will be approved soon by the FDA are expected to move from Project BioShield to the Strategic National Stockpile for

future acquisition; these include anthrax and botulinum antitoxins and smallpox vaccines and antiviral drugs. As multiyear budgeting improves within the PHEMCE, these transitions will become seamless and able to address life cost management more effectively.

The PHEMCE and BARDA still face formidable obstacles in the coming years including complacency towards biodefense threats; emergence of new threats, especially from Mother Nature; managing life cycle costs of existing and more new products; new regulatory pathways to traverse; and the uncertainty of funding to purchase new medical countermeasures under Project BioShield to maintain and enhance preparedness and readiness levels. BARDA will tackle these challenges with PHEMCE and industry partners by executing existing plans for building domestic manufacturing capacity and streamlining development costs through the CIADMs and working with FDA to establish regulatory management plans for product developers. In conclusion, I would like to reiterate that as a member of the PHEMCE, BARDA is a proven and reliable partner and is creating vital national assets that enhance national security and address public health preparedness and response needs. Again, I would like to thank the subcommittee for the opportunity to testify, and I look forward to your questions.