

DEPARTMENT OF HEALTH AND HUMAN SERVICES
NATIONAL INSTITUTES OF HEALTH

**The Role of NIH Research
In The
Public Health Emergency Medical Countermeasures Enterprise**

Statement for the Record

House Subcommittee on Labor-HHS-Education Appropriations

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Mr. Chairman and Members of the Committee:

Thank you for the opportunity to discuss the role of the National Institutes of Health (NIH) in the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE). I am the Director of the Office of Biodefense, Research Resources, and Translational Research, and also serve as the Associate Director for Biodefense Product Development at the National Institute of Allergy and Infectious Diseases (NIAID). NIAID is the component of the NIH with the lead for research on biodefense and emerging and re-emerging infectious diseases. In fiscal year 2013, NIH funding for biodefense and emerging infectious diseases was approximately \$1.7 billion.

OVERVIEW

The September 11, 2001, terrorist attacks and anthrax attacks in the fall of 2001 underscored the importance of a national strategy for the development of medical countermeasures to combat bioterrorism and naturally emerging or re-emerging disease threats. To address this challenge, the NIH supports foundational research toward the development of new and improved medical countermeasures against biological, chemical, radiological, and nuclear threats, including emerging infectious diseases. NIH collaborations with its PHEMCE partners are critical to this endeavor. The NIH holds senior leadership positions within the PHEMCE, and participates throughout all its levels. For example, NIH subject matter experts provide input to all Integrated Program Teams (IPTs) that coordinate efforts on particular biodefense threats, and chair several IPTs.

Since 2001, NIAID has greatly accelerated its biodefense research program to rapidly respond to known and possible future threats, whether man-made or naturally occurring. NIH research addresses potential attacks using microbes, toxins, chemical agents, and radiation, as well as naturally occurring emerging or re-emerging infectious disease threats such as seasonal and pandemic influenza. As part of this effort, NIAID conducts and supports basic research on microbiology and immunology; applied research to develop medical countermeasures; and clinical research to evaluate candidate diagnostics, therapeutics, and vaccines. NIAID coordinates with industry and PHEMCE partners to ensure that the results of NIAID-supported research can be translated rapidly into safe and effective medical countermeasures. In particular, NIAID transitions the advanced research and development of high-priority medical countermeasures to the Biomedical Advanced Research and Development Authority (BARDA) with the eventual goal of Food and Drug Administration (FDA) approval, licensure, clearance, or authorization and possible inclusion in the Strategic National Stockpile.

MEDICAL COUNTERMEASURES DEVELOPMENT:

RECENT ACTIVITIES AND ADVANCES

Biodefense and Emerging and Re-emerging Infectious Diseases. NIAID supports research and early-stage development of medical countermeasures against bioterror threats and emerging and re-emerging infectious diseases of public health importance. NIAID maximizes its efforts by pursuing development of medical countermeasures with wide impact, such as broad-spectrum antibiotics and antiviral drugs effective against

multiple bacteria or viruses. NIAID also seeks to establish and validate efficient platform technologies to more rapidly develop vaccines and diagnostics for a variety of threat agents. NIAID's migration from a "one bug, one drug" approach toward a broader, more flexible research paradigm is yielding scientific advances that will facilitate public health emergency preparedness and our ability to respond to emerging public health threats.

Recent successes in NIAID's biodefense program include early-stage development of a next-generation smallpox vaccine. This vaccine would be suitable for populations, such as immunocompromised individuals, for whom the currently licensed smallpox vaccine is contraindicated. NIAID is currently exploring the use of this vaccine as a potential platform to facilitate the development of other vaccines. This next-generation smallpox vaccine and two smallpox antiviral drug candidates have been transitioned to BARDA for further development. Other candidate products recently transitioned from NIAID to BARDA include therapies for anthrax and pandemic influenza, and a next-generation anthrax vaccine. NIAID has conducted a study of the currently licensed anthrax vaccine to inform policy makers on strategies to extend vaccine supply should a shortfall occur. NIAID also successfully conducted studies that supported the first antibiotic approvals for pneumonic plague, a form of plague that causes severe and deadly lung infections, under the FDA's Animal Rule.

Influenza. Seasonal influenza and the possibility of an emerging influenza pandemic remain a serious public health challenge. The Centers for Disease Control and Prevention (CDC) estimates that seasonal influenza causes up to 49,000 deaths in the

United States annually. Worldwide, the World Health Organization estimates that each year seasonal influenza causes up to 500,000 deaths.

NIAID supports and conducts research to better understand this global threat and develop medical countermeasures to diagnose, treat, and prevent seasonal and pandemic influenza. NIAID's efforts span the product development pipeline from basic research to understand influenza pathogenesis through discovery and testing of improved influenza antiviral therapeutics and vaccines. NIAID influenza research contributes to preparedness for emerging strains and potential pandemics. For example, NIAID collaborated with CDC, FDA, and BARDA to rapidly develop a vaccine for 2009 H1N1 pandemic influenza. More recently, NIAID efforts have focused on the evaluation of H7N9 avian influenza vaccine candidates. In addition, NIAID is leading efforts to develop a so-called "universal" influenza vaccine to protect against multiple strains of seasonal and pandemic influenza. Such a vaccine could do away with the need for an annual vaccination and potentially save millions of lives and benefit global economic productivity.

Radiological/Nuclear and Chemical Threat Agents. NIAID research aims to understand the effects of radiation on the body, such as damage to the bone marrow, gastrointestinal tract, and lungs, and to develop medical countermeasures to diagnose and treat radiation exposure. To date, twenty radiological/nuclear medical countermeasure candidates and six biodosimetry approaches supported by NIAID have advanced to BARDA for further development. In addition, NIAID has funded animal efficacy studies of Neupogen for the treatment of hematopoietic acute radiation

syndrome. A supplemental Biologics License Application for FDA review is in preparation.

The NIH medical chemical defense program, a trans-NIH partnership that includes NIAID, the National Institute of Neurological Disorders and Stroke (NINDS), the National Institute of Environmental Health Sciences, and a number of other NIH Institutes and Centers, is closely coordinated with PHEMCE partners and supports research to enhance protection against exposure to dangerous chemicals including nerve agents, metabolic poisons, and toxic industrial chemicals. Notably, NINDS recently supported clinical trials demonstrating that the drug midazolam may be an effective treatment for exposure to nerve agents for adults, as well as children. The results of these trials provide crucial support for midazolam as a medical countermeasure against nerve agents. Medical countermeasures addressing other significant chemical hazards are under development, including drugs to treat pulmonary complications and neurotoxic effects of these chemicals.

CONCLUSION

The NIH remains committed to meeting public health emergency needs by advancing high-priority research for the development of medical countermeasures to combat pathogens utilized as bioterrorism agents and naturally emerging or re-emerging disease threats and chemical and radiological agents. Together with our PHEMCE partners, the NIH will continue to pursue the development of diagnostics, therapeutics, and vaccines that will increase our national preparedness.