INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

Forum on Medical and Public Health Preparedness to Catastrophic Events
Forum on Drug Discovery, Development, and Translation
Forum on Microbial Threats

Board on Health Sciences Policy
Board on Global Health

Enabling Rapid Response and Sustained Capability with Medical Countermeasures to Mitigate Risk of Emerging Infectious Diseases: An Institute of Medicine Workshop

AGENDA

March 26-27, 2015

Room 125 of the National Academy of Sciences Building
2101 Constitution Avenue, N.W., Washington, DC 20418

Background:

Ensuring ready access to medical countermeasures (MCMs) for emerging infectious diseases such as pandemic influenza has been an issue accumulating national attention. With the 2013 emergence and persistence of the H7N9 pandemic influenza threat, the 2014 Ebola outbreak affecting West Africa and several countries around the globe, and a recent surge in cases of MERS-CoV in the Middle East, the real and present danger of these emerging infectious diseases, which know no borders, are increasingly a national security issue. As the National Health Security Strategy states, "As the movement of people, goods, and services across borders increases, our national health security is increasingly dependent on global health security". Though similar in some ways to intentional biological threats like anthrax, these naturally occurring threats present a unique challenge to the medical countermeasures enterprise given the persistent, dynamic and unpredictable nature of their epidemiological trajectories. Traditional means of risk assessment and mitigation may require novel approaches. Is rapid response with MCMs a reality now or in the future, if the MCM is not already in advanced development or available in stockpiles? Are public-private partnerships well positioned to respond in a timely manner?

Current operational and business models to build and sustain this capability are limited. Secure multiyear markets, a fundamental tenet of the public-private partnership for MCM advanced development and acquisition programs for intentional CBRN threats (i.e., Project BioShield) and the initial phase of pandemic preparedness have been dramatically reduced. In a Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) now dependent on annual appropriations, and with limited funding to support rapid response as evidenced in 2014 with the Ebola outbreak, these models face new challenges. The PHEMCE in 2009 proposed new strategies and approaches to medical countermeasure development. However, it did not solve all challenges, and recent decreases in funding and shifts to annual appropriations may prove to negate some initial successes. In between events such as H1N1 Influenza outbreaks, mission capabilities need to be sustained so capacity is not lost when the next event emerges. Additionally, many regulations and policies have been developed in response to past events,

instead of looking forward to potential future needs and creating capabilities and partnerships in a systematic manner.

This workshop, hosted by the Forum on Medical and Public Health Preparedness for Catastrophic Events, the Forum on Drug Discovery, Development, and Translation, and the Forum on Microbial Threats will bring together public and private sector stakeholders to discuss how to achieve rapid and nimble medical countermeasure availability for new and emerging threats. Discussions will include real-world case studies to elucidate how past events were handled from a policy, budget, and operational standpoint, and contribute to a better aggregate picture of what capabilities and resources are needed moving forward.

Meeting Objectives:

- Discuss the Nation's capacity to provide rapid access to medical countermeasures (MCMs) for EIDs, delineate preparedness gaps, and identify activities required by all stakeholders to improve capabilities
    - Consider the impact of the current fiscal environment and reasonable expectations
    - Examine the sustainability of public-private partnerships
- Examine the role of MCMs for emerging infectious disease threats as a national security issue
    - Discuss the ethical, economic, and global dimensions of these threats and the public-private partnerships required to establish robust capabilities.
- Discuss case studies of past incidents of emerging threats to understand government and private sector decisions and lessons learned.
    - Evaluate potential strategies for rapid availability of needed MCMs; examine the operational and business models required to enable post-event rapid development, translation, and response in terms of regulatory pathways, financing and market opportunities, and the value proposition to private sector partners.
    - Discuss the integration of "One Health" efforts into ongoing threat assessments prior to a declared emergency.
- Consider how to operationalize next steps for the public and private sector to coordinate a more rapid and nimble response to global emerging threats.
    - Discuss common elements across a range of threats
    - Consider the sustainability of business models to keep stakeholders invested

Enabling Rapid Response and Sustained Capability with Medical Countermeasures to Mitigate Risk of Emerging Infectious Diseases: An Institute of Medicine Workshop

AGENDA

March 26, 2015

Room 125 of the National Academy of Sciences Building
2101 Constitution Avenue, N.W., Washington, DC 20418

8:30a.m. Welcome and Introductions: *Workshop co-chairs*

RICHARD HATCHETT
Chief Medical Officer, Deputy Director
Biomedical Advanced Research and
Development (HHS/ASPR/BARDA)

MONIQUE K. MANSOURA
Head, Medical Countermeasures &
Government Affairs, Americas
Novartis Influenza Vaccines

8:35 a.m. Global Health Security Initiative Update

VICTOR DZAU
President
Institute of Medicine

8:55 a.m. Opening Keynote:

JEREMY FARRAR
Director
Wellcome Trust

SESSION I: Framing Preparedness for Emerging Infectious Diseases as a National Security Imperative

Session Objectives:

- Overview the current ability to have MCMs available to effectively respond to EIDs of high (national security) impact
- Discuss budgetary and policy issues that have challenged rapid and nimble response
- Identify barriers that might prevent a prospective framework for managing future needs

9:15 a.m. **Session Chair: Introduction and Overview of Objectives**

GERALD W. PARKER, Vice President, Public Health Preparedness and Response, Texas A&M Health Science Center

9:25 a.m. **Panel Discussion: Rapid MCM Response as a National Security Imperative**

National Security: **ANDREW C. WEBER**, Deputy Coordinator for Ebola Response, Department of State

Biosecurity Strategy: **TARA O'TOOLE,** Senior Fellow, In-Q-Tel, Inc.

Department of Defense/Warfighter Perspective: **CDR FRANCA JONES**, Medical Director, Office of the Deputy Assistant Secretary of Defense for Chemical and Biological Defense Programs

Economics: **ROBERT SHAPIRO**, Cofounder and Chairman, Sonecon, LLC, former Undersecretary of Commerce for Economic Affairs

Global Risk Report: **SURESH KUMAR**, Senior Partner, Oliver Wyman Public Sector and Health & Life Sciences Practice, Marsh & McLennan Companies

Industry perspective: **DANIEL J. ABDUN-NABI**, President and CEO, Emergent BioSolutions Inc.

Ethical Considerations: **LISA M. LEE**, Executive Director, Presidential Commission for the Study of Bioethical Issues

10:40 a.m. BREAK

11:00 a.m. Facilitated Discussion with Attendees *(1hr)*

Potential Discussion Questions "In your experience…":

- *What is the cost and social impact of inaction and remaining reactionary?*
- *How to bridge the gap between the value to society versus the value to the industry?*
- *What high level budget and policy gaps do you see that present a challenge to rapid response?*
- *How could international collaboration support rapid response?*

12:00 p.m. U.S. Preparedness Perspective: Sustainability and Threat Assessment

NICOLE LURIE
Assistant Secretary for Preparedness and Response
Department of Health and Human Services

12:30 p.m. LUNCH

SESSION II: 2014 Ebola Outbreak Response

Session Objectives:

- Discuss methods and findings of recently released Ebola Team B report
- Discuss budgetary and policy issues that have challenged rapid and nimble response
- Highlight issues that have caused fast-acting companies to withdraw from response efforts
    - How can partners think creatively to demonstrate efficacy when disease incidence decreases?

1:15 p.m. **Session Chair: Introduction and Overview of Objectives**

MICHAEL T. OSTERHOLM, McKnight Presidential Endowed Chair in Public Health Director, Center for Infectious Disease Research & Policy (CIDRAP) University of Minnesota

1:25 p.m. **Panel Discussion: Fast Track Development of Ebola Vaccines and Testing: Reviewing Successes and Understanding Remaining Barriers to a "Rapid" Response in Real Time**

ROBIN ROBINSON, Director, Biomedical Advanced Research and Development Authority (BARDA)

MARK FEINBERG, Vice President and Chief Public Health and Science Officer, Merck Vaccines

MARION GRUBER, Director, Office of Vaccines Research and Review, Center for Biologics Evaluation and Research, Food and Drug Administration

WOUTER LATOUR, Chief Executive Officer, Vaxart

THOMAS A. DUNN, Program Manager for the Next Generation Diagnostics System Increment 1, Joint Project Management Office for Medical Countermeasures Systems (JPM-MCS)

2:15 p.m. Facilitated Discussion with Attendees *(1 hr)*

"In your experience…":

- *How did the uncertainty about the market impact your investment decision?*
    - *What guarantees would you need to move forward? Who can be looked to for purchasing and continuity once they are finalized?*
- *What did you wish was in place ahead of the response or while it unfolded?*
    - *Were there components of the response that worked well compared to prior emerging threat situations?*

- *How did the evolving ethical considerations in performing clinical trials impact your decisions?*
    - *Do we need an ethical framework for questions about allocation of scarce investigational agents?*
- *How did the existing regulatory framework influence decisions regarding your clinical trials?*
- *Knowing that cases are winding down and clinical trials will be difficult to execute, how will that impact future development or motivation to respond?*
    - *What does this mean for the products now in development?*
    - *Will these products become registered?*
    - *How will any registered products sustained post-registration? Who will manage the supply chain? Who will manage pharmacovigilance? Who will drive registration in the relevant territories?*

3:15 p.m. BREAK

SESSION III: Pandemic Influenza

Session Objectives:

- Discuss policy, budgetary and operational challenges for companies to continue work in pandemic influenza countermeasure development.
- Explore risk assessment needs and opportunities for current tools and frameworks
- Consider needs for balancing a rapid timeline with sustainable stockpiles
    - How fast is "rapid"?
    - What must be stockpiled and what can wait?

3:30 p.m. **Session Chair: Introduction and Overview of Objectives**

ANDREW PAVIA, George and Esther Gross Presidential Professor Chief Division of Pediatric Infectious Diseases, University of Utah

3:40 p.m. **Panel Discussion: Opportunities and Challenges in Preparedness and Response to Pandemic Influenza Threats**

JACQUELINE KATZ, Deputy Director (acting) Influenza Division
National Center for Immunization and Respiratory Diseases
Centers for Disease Control and Prevention (CDC)

RICK BRIGHT, Director of the Influenza Division, Biomedical Advancement Research and Development Authority (BARDA)

LOUIS FRIES III, Vice President, Chief Medical Officer, Novavax

MONIQUE K. MANSOURA, Head, Medical Countermeasures & Government Affairs, Americas, Novartis Influenza Vaccines

DAVID W. VAUGHN, Head of External R&D, North America, GSK Vaccines

4:30 p.m. Facilitated Discussion with Attendees *(1 hr)*
"In your experience…":

- *What are the questions that are asked when making decisions? What factors impact each of those decisions?*
- *What regulations challenges have you encountered during development?*
- *What financing strategies are needed to enable continued capacity to rapidly produce new anti-viral countermeasures?*
- *How fast is "rapid"? What must be stockpiled and what can wait?*
- *How do you sustain development and production of stockpiled products?*
    - *Does this process change once a threat has been reduced or eliminated?*

5:30 p.m. ADJOURN

Enabling Rapid Response and Sustained Capability with Medical Countermeasures to Mitigate Risk of Emerging Infectious Diseases: An Institute of Medicine Workshop

AGENDA

March 27, 2015

Room 125 of the National Academy of Sciences Building
2101 Constitution Avenue, N.W., Washington, DC 20418

8:30a.m. Welcome and Introductions

RICHARD HATCHETT, *workshop co-chair*
Chief Medical Officer and Deputy Director
Biomedical Advanced Research and Development Authority
(HHS/ASPR/BARDA)

MONIQUE K. MANSOURA, *workshop co-chair*
Head, Medical Countermeasures & Government Affairs, Americas
Novartis Influenza Vaccines

8:35 a.m. Opening Remarks

RAJEEV VENKAYYA
President, Global Vaccine Business Unit
Takeda Pharmaceuticals

SESSION IV: Developing Vaccines and Therapeutics to Emerging Infectious Diseases Prior to the Emergency Threshold: Coronaviruses

Session Objectives:

- Consider the implications of negative funding following the 2003 SARS outbreak
- Discuss the need for ongoing threat and risk assessments and implications for MCM development before an outbreak reaches public health emergency threshold.
- Discuss the integration of One Health Initiative efforts prior to diseases reaching a public health emergency threshold

9:00 a.m. **Session Chair: Introduction and Overview of Objectives** (*10 min)*

TOM INGLESBY, Director, UPMC Center for Health Security

9:10 a.m. **Panel Discussion: Challenges and Opportunities in Responding to Coronaviruses: Past and Present** *(10 min each)*

DAVID SWERDLOW, the Associate Director for Science, National Center for Immunization and Respiratory Diseases (NCIRD), CDC

FRED CASSELS, former SARS Program Officer, current Chief of Enteric and Hepatic Diseases Branch, Division of Microbiology and Infectious Diseases, NIAID

JEFFREY ULMER, Global Head, External Research, GSK Vaccines

MICHAEL WONG, Senior Medical Director for Infectious Diseases, Sarepta Therapeutics

PETER DASZAK, President, EcoHealth Alliance

10:00 a.m. BREAK

10:20 a.m. Facilitated Discussion with Attendees *(1hr)*

"In your experience…":

- *What are the questions that are asked when making decisions? What factors impact each of those decisions?*
- *Can we anticipate future regulatory needs for unknown agents?*
- *Are new financing vehicles necessary to enable rapid response to emerging threats?*
- *When there are multiple issues to worry about, how can you prioritize which diseases and countermeasures should get attention and funding?*
- *How can threat assessments be improved so decision support exists on when to move forward and when to wait?*
- *How can you minimize the risk to existing programs in the face of re-prioritization of resources for an escalating threat?*

11:20 a.m. LUNCH

SESSION V: Sustainability and Maintenance of Business Models to Ensure Rapid and Nimble Response to Emerging Threats of National Security Concern

Session Objectives:

- Discuss internal and external ideas to mounting a rapid response to emerging threats that are presented by current business models and public private partnerships (PPPs) in other sectors.
    - Consider opportunities for companies to collaborate in pre-competitive areas and solve anti-trust issues and perceptions

- Discuss ways to better leverage PPPs for future emerging threats
- Consider how to operationalize next steps for the public and private sector to coordinate a more rapid and nimble response to global emerging threats.
    - Discuss common elements across a range of threats
    - Consider the sustainability of business models to keep stakeholders invested

12:20 p.m. **Session Chair: Introduction and Overview of Objectives**

JOHN REX, Senior Vice President and Head of Infection, Global Medicines Development, Astrazeneca

12:30 p.m. **Panel Discussion: Lessons Learned from Sectors and Potential Future Strategies for Infectious Diseases of National Security Concern** *(5-10 min each)*

Proposed Speakers:

JOE LARSEN, Deputy Director, Division of CBRN Medical Countermeasures, Biomedical Advanced Research Development Authority (BARDA)

ROBERT GARRY, Program Manager, Viral Hemorrhagic Fever Consortium, Tulane University

MEGHAN MAJOROWSKI, Global Health Director, FSG

DEBRA HANNA, Executive Director for the Critical Path to TB Drug Regimens Consortium, Critical Path Institute

1:10p.m. Facilitated Discussion with Attendees *(1hr)*

"In your experience…":

- *What is needed to ensure that business models are more resilient to the inherent uncertainly associated with potential MCM markets?*
- *Where are policies needed to improve market conditions for industry investment?*
- *Are there business models from other industries that are relevant?*
    - *How do we maintain the discovery apparatus? Would real-world challenges with priority pathogens be a way to sustain interest and focus?*
    - *How do we maintain registered products? Please address issues of post-registration commitments, supply chain maintenance, global registration (and regulatory maintenance), and pharmacovigilance.*
    - *How should these products be valued? What is the correct reward model?*

- *What regulatory policies have influenced sustainability of alternative business models?*
- *What are strategies for managing both sides of the risk/benefit equation for public and private partners?*
    - *How can the perceived value to society be separated from the perceived value to market?*
- *How do you sustain this new priority area?*

2:10 p.m. BREAK

2:30 p.m. **Response Panel: Report out from Discussions – Common elements and priorities identified across threats**
Evaluate potential strategies in terms of regulatory pathways, financing and market opportunities, and value proposition to private sector partners. How can we get to the next level?

Session Chairs:

GERALD W. PARKER, Texas A&M Health Science Center

MICHAEL T. OSTERHOLM, CIDRAP

ANDREW PAVIA, University of Utah

TOM INGLESBY, UPMC Center for Health Security

JOHN REX, Astrazeneca

3:15 p.m. Discussion with Attendees:

Potential Discussion Questions:

- *How can we create policies to support MCM development across threat levels, countermeasure types, and funding cycles?*
- *How can business models be created to engage the private sector and maintain interest of stakeholders across the MCM response spectrum?*

4:15 p.m. Next Steps: Key takeaway messages

4:30 p.m. ADJOURN