BARDA Industry Day: East Coast 2011

Sheraton Boston

Robin Robinson, Ph.D.
Director, BARDA
Deputy Assistant Secretary, ASPR

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BARDA Mission

Develop and provide countermeasures for CBRN threats, pandemic influenza, and emerging infectious diseases by product development, stockpile acquisition, manufacturing infrastructure building, and product innovation.

• Unique niche in USG biomedical R&D
  — Bridge the “Valley of Death”
  — Mid- to late-stage product development
  — Experienced TEAM in product development, regulatory, clinical, and manufacturing
  — Work with industry to facilitate candidates through development pipeline
  — Response capability for product manufacturing
Medical Countermeasure Development is Expensive, Lengthy and High Risk

• Industry benchmarks for typical drug
  – 10-15 yrs from bench to pharmacy
  – Phase 2 to licensure 6-8 yrs
  – $800M to $1B investment
  – Very low probability of success

• MCM development characterized by market failure

• USG provides numerous incentives to attract private sector partners to medical countermeasure development
  – Grants and contracts provided by NIH, BARDA, and DoD
  – Drug screening, preclinical, clinical, and regulatory services
  – Project BioShield Special Reserve Fund for procurement of needed MCMs (market guarantee)
CBRN Countermeasure Development Is Risky & Expensive

- NIH ($12.7B)
- BARDA ($786M)
- Project BioShield ($5.6B)

**Valley of Death**

- **Discovery**
- **Preclinical Development**
- **Phase I**
- **Phase II**
- **Phase III**
- **Licensure**
- **Production & Delivery**

**Product Pipeline**

- **Time**:
  - 3-7 yr
  - 0.5-2 yr
  - 1-2 yr
  - 2-3.5 yr
  - 2.5-4 yr
  - 1-2 yrs

- **Phase Cost**:
  - $100M - $130M
  - $60-70M
  - $70M-100M
  - $130M-160M
  - $190M-220M
  - $18M-20M

**Probability of Success to Licensure**

- 1-3%
- 5-17%
- 10-25%
- 18-35%
- 45-70%
- 90%

**Valley of Death** is a term used to describe the high risk and high cost associated with the development of countermeasures for CBRN (Chemical, Biological, Radiological, and Nuclear) threats. The diagram illustrates the phases of development, from discovery to licensure, and the associated risks and costs. The probability of success at each phase is also indicated.
History of Biodefense & BARDA

• pre-9/11, 1950s – 2001
• post-9/11 to Project BioShield, 2001 – 2004
• Project BioShield and PAHPA, 2004 – 2006
• ASPR, BARDA & H1N1, 2007 – 2009
• Enterprise Transformation, 2010 →
Public Health Emergency Medical Countermeasures Enterprise (PHEMCE)

Other Members:

ASPR/OPP  NIH  ASPR/BARDA  ASPR/BARDA  CDC/SNS  BARDA  CDC and ASPR/OPEO  CDC ASPR

FDA

Requirements  R & D  Advanced Development  Acquisition & Stockpiling  Storage/Maintenance  Deployment  Evaluation

PHEMCE COORDINATED PLANNING & EXECUTION
PHEMCE Process For Developing Medical Countermeasure Requirements and Acquisition Strategies

Pillar 1
Identify and Assess Threats

Pillar 2
Evaluate Medical and

Pillar 3
Establish and Prioritize Medical Countermeasure Requirements

Pillar 4
Determine Near-, Mid-, and Long-Term Development & Acquisition Strategies
Material Threat Determinations and Population Threat Assessments Issued by the Department of Homeland Security

- Radiological and Nuclear agents
- *Bacillus anthracis* (anthrax)
- *Bacillus anthracis* – Multi-drug resistant (MDR anthrax)
- Botulinum toxins (botulism)
- Variola virus (smallpox)
- *Yersinia pestis* (plague)
- *Francisella tularensis* (tularemia)
- Hemorrhagic Fever Viruses - Ebola, Marburg
- *Burkholderia mallei* (glanders)
- *Burkholderia pseudomallei* (melioidosis)
- *Rickettsia prowazekii* (typhus)
- Low volatility and volatile nerve agents
- Hydrogen, potassium, and sodium cyanide
Integrated Portfolio for CBRN MCM Requirements – Unique and Convergent

DoD focus is on protecting forces prior to exposure. HHS focus is on response to threats to general civilian population after exposure

**DoD-Unique**
- Brucellosis Vx
- VEE/EEE/WEE Vx & Rx
- Plague Vx
- Botulism Vx
- SEB Vx & Rx
- Tularemia Vx
- Ricin Vx & Rx
- (other, unfunded)

**HHS-Unique**
- Smallpox Vx for special populations
- Burkholderia sp. Rx
- Junin Rx
- Plague Rx

**Common**
- Anthrax Vx & Rx
- Smallpox Vx & Rx
- Ebola / Marburg Vx & Rx
- Tularemia Rx
- Botulism Rx
- Radiation Rx
- Nerve agent Vx & Rx

Vx = Prophylaxis  Rx = Therapeutic
BARDA Threats & Projects: Facts & Figures

• BARDA portfolios address serious threats
  – Anthrax, smallpox, plague, tularemia, botulism, melioidosis, glanders, typhus, viral hemorrhagic fever
  – Acute radiation syndrome & thermal burns
  – Volatile chemical agents
  – Pandemic & seasonal influenza
  – Emerging infectious pathogens incl. antimicrobial resistance

• BARDA portfolios include multiple countermeasure types
  – Vaccines
  – Small molecule therapeutics
  – Biological therapeutics
  – Diagnostics & medical devices

• BARDA supports 90+ candidates for advanced development of pandemic influenza and CBRN medical countermeasures

• BARDA is # 5 investor globally in pharmaceutical product R&D (>3 B since 2005)
Public – Private Partnership Framework

• BARDA & Industry Areas of Interest
  — Product advanced development
  — Stockpiling
  — Manufacturing infrastructure – Vaccine Mfg Facilities

• Cost-sharing

• Long-term contractual relationships

• Address government & commercial needs
  — Domestic & global emergency preparedness & response
  — Commercial markets
  — Public health needs – Broad Spectrum Antimicrobials
Key Initiatives

1. Expand Product Pipeline through Concept Acceleration Program (CAP) at NIAID

2. Establish a Strategic Investment (SI) Fund to increase investments in commercial ventures with multi-use potential (BARDA)

3. Establish Centers for Innovation in Advanced Development and Manufacturing (BARDA)

4. Investment in upgrading science capacity at FDA

5. Optimize influenza vaccine development and manufacturing (BARDA. NIAID. FDA. CDC)

Enhancements:
• Business Practices
BARDA Strategic Plan
2011-2016

• Five (5) major goals
• Strategic approaches
• Cascades from NHSS, ASPR, MCM Review, and other national plans
• View Plan on medicalcountermeasures.gov
BARDA Strategic Goal 1

• An advanced development pipeline replete with medical countermeasures and platforms to address unmet public health needs, emphasizing innovation, flexibility, multi-purpose and broad spectrum application, and long-term sustainability
BARDA Strategic Goal 2

• A capability base to provide enabling core services to medical countermeasure innovators
BARDA Strategic Goal 3

• Agile, robust and sustainable U.S. manufacturing infrastructure capable of rapidly producing vaccines and other biologics against pandemic influenza and other emerging threats
BARDA Strategic Goal 4

• Responsive and nimble programs and capabilities to address novel and emerging threats
BARDA Strategic Goal 5

• A ready capability to develop, manufacture and facilitate distribution of medical countermeasures during public health emergencies
BARDA FY 2011 Major Accomplishments

• Expanded BARDA CBRN & Flu AD programs to 90+ product candidates
• Awarded 1st PBS & ARD contracts for antiviral drugs - smallpox
• Awarded 1st CBRN contract to major pharmaceutical company - BSA
• Awarded 1st contract to develop chemical antidotes
• Developing formulations for Rad/Nuc chelators for children
• Preserved botulinum antitoxin stockpile through 2026
• With CDC, two (2) influenza diagnostics were FDA-approved
• Faster sterility assay accepted by FDA for influenza vaccine mfg.
• New cell-based influenza vaccine facility became operational for pandemics & intl. vaccine capacity in 10+ developing countries
• Awarded contracts for recombinant-based influenza vaccine and longer, lasting NAI development
• Began CIADM program with contract solicitation
• Established animal studies network
New cell-based influenza vaccine mfg. facilities (Novartis): Operational Nov. 2011
BARDA
Milestones & Challenges

• PAHPA Reauthorization
• Economic Woes & Budget Austerity
• Pharmaceutical & Biotech Industry Reinvention
• Special Populations
• Re-purposing Products
• Stockpile Replenishment
• Core Service Assistance
• Program Transitioning
BARDA Core Services

Regulatory & Clinical Affairs

Technical Expertise

Animal Studies Network

Strategic Investor

Fill Finish Mfg Network

Centers for Innovation in Advanced Development & Manufacturing
BARDA Program Transitioning

• **Completion of mature programs**
  – Cell-based influenza vaccines
  – Botulinum antitoxins
  – Smallpox Vaccines

• **Moving programs from mid-stage to maturity**
  – Next Generation Anthrax Vaccines & Antitoxins
  – Smallpox Antivirals
  – Antigen-sparing Influenza Vaccines
  – NAI Influenza Antivirals, Diagnostics, & Ventilators

• **Kicking off infant programs into growth phase**
  – Broad Spectrum Antimicrobials
  – Rad/Nuc Therapeutics
  – Biodosimetry Devices
  – Chemical Antidotes
  – Recombinant Influenza Vaccines

• **Starting new programs**
  – Biothreat Diagnostics
  – Non-NAI Antivirals & Small Molecule Immune Modulators
  – “Universal “ Influenza Vaccines ????
Contact Us

BARDA:
URL: http://www.phe.gov/about/barda
E-Mail: BARDA@hhs.gov

• Upcoming Events
• PHEMCE Strategy and Implementation Plan
• CBRN and Pan Flu Programs

MedicalCountermeasures.gov

• Tech Watch program
• Federally-sponsored conferences
• Funding opportunities
• Resources 7 core service programs
• Regulatory guidance
• Federal strategies and reports