Development and Procurement of Biotechnology for Emerging Disease and Engineered Threats in the Public Health Preparedness Sector

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Public Health Preparedness Funding
Key Considerations

How to Pursue Non-Dilutive Funding
• What does it take and how long?

Proposals and Requirements
• The government “red tape”

Commercial Strategy
• How does non-dilutive funding fit with your business strategy?

Alternative Sources
• Working with charities and foundations

Collaboration Maintenance
• Maintaining relationships with non-dilutive funding partners
Public Health Preparedness Spending
US Government

- HHS/ASPR/BARDA
  - Project BioShield
  - Pandemic Flu
  - Advanced Research and Development

- NIAID/NIH

- DoD
  - DARPA

- CDC

- FDA

- USDA

- DHS
  - DHS S&T
  - Office of Health Affairs

- EPA

- JPEO-CBMS
Global Public Health Initiatives
Scope of Opportunity

Governments
- US
- EU
- UK
- DFID

Foundations
- Rockefeller Foundation
- Wellcome Trust
- Alfred P. Sloan Foundation
- FNIH

Global Public Health Initiatives
- Gates Global Health Program

Non-Gov. Orgs.
- Global Fund
- GAVI
- IFFIm
- ICDDR,B
- TDR
- UNICEF
- WHO
- UN
- PAHO
- MVI
- PATH
- UN

Academic Research Institutions
- Broad Institute
- IDRI
- SVI
- SBRI
- UNC CPDD

Product Development Partnerships
- PDVI
- TB Alliance
- FIND
- DNDi
- IPM
- MMV
- AERAS
- IAVI
- IOWH
- MVI
- PATH

Strategic Coalitions
- UK Partnership For Global Health
- EPHG
- GHSI
Where’s the Money in Infectious Disease?
## Where’s the Money in Infectious Disease?

<table>
<thead>
<tr>
<th>Agency or Activity</th>
<th>Amount of Funds</th>
<th>Timeframe</th>
<th>Function of Funds</th>
</tr>
</thead>
<tbody>
<tr>
<td>BioShield/HHS</td>
<td>$2.8 Billion ($5.6 B from 2004-2010)</td>
<td>5 Years</td>
<td>Procurement of medical countermeasures</td>
</tr>
<tr>
<td>H5N1/H1N1 Influenza Preparedness</td>
<td>$6.1 Billion (H5); $5 billion, plus (H1); $100 million, plus Universal Vaccines</td>
<td></td>
<td>Pandemic preparedness funding (includes advanced development and procurement of influenza vaccines, therapeutics, devices, and diagnostics)</td>
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<tr>
<td>BARDA Advanced Development</td>
<td>$400 million plus</td>
<td>Annual</td>
<td>Advanced development of medical countermeasures</td>
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<tr>
<td>DOD</td>
<td>$500 Million</td>
<td>Annual</td>
<td>Development and procurement of medical countermeasures</td>
</tr>
<tr>
<td>NIH/NIAID</td>
<td>$32 Billion/$1.79 Billion</td>
<td>Annual</td>
<td>Development of pre-clinical medical countermeasures</td>
</tr>
<tr>
<td>DHS</td>
<td>$12 Million</td>
<td>Annual</td>
<td>Disaster preparedness planning</td>
</tr>
<tr>
<td><strong>TOTAL</strong></td>
<td><strong>$20 Billion plus</strong></td>
<td></td>
<td><strong>TOTAL</strong></td>
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HHS Public Health Enterprise

The Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) coordinates intra-agency effort with mission to define and prioritize requirements for public health medical emergency countermeasures

- Assistant Secretary Preparedness and Response (ASPR)
  - The ASPR Office was created under the Pandemic and All Hazards Preparedness Act (PAHPA) to lead the nation in preparing for, preventing and responding to public health emergencies and disasters

- Biomedical Advanced Research and Development Authority (BARDA)
  - BARDA manages the Procurement of MCMs under Project BioShield and directs the Advanced Development of a pipeline of MCMs for chemical, biological, radiological, and nuclear agents
  - Project BioShield
    - A comprehensive effort during the George W. Bush Administration resulting in a $5.6B strategic reserve fund to Procure and Develop drugs and vaccines to protect against attack by chemical, biological, radiological, and nuclear agents
Non-Dilutive Funding Strategy

- Another BD target with unique needs
- Map technological benefits with government requirements
- Create realistic timelines for success
- Advocacy and opportunity sourcing occurs in many forums:
  - Direct interaction with decision-makers
  - Participation in industry meetings and trade associations
  - Participation in media sessions and investor conferences
  - Online outlets and through the blogosphere
- Establish and nurture resilient relationships with relevant non-government players
  - Industry collaborators and partners
  - Non-Governmental Organizations
  - Media outlets
Non-Dilutive Funding Strategy

- Successful government strategies MUST make full use of global alliances and networks
- Significant funding opportunities exist across the USG and beyond the USG
  - Different agencies
  - NGOs
  - Charities
  - Other governments

The USG expects you to know this and exploit this!
Broad Non-Dilutive Funding Strategy

Governments increasingly collaborating with each other as well as Non-Governmental Organization, such as:

– UK Defence Science and Technology Lab (DSTL)
– Public Health England (PHE)
  • Biodefense
  • Community and hospital acquired infections (HAI)
– European Centers for Disease Control in HAI monitoring
– Gates Foundation and World Health Organization
  • Global public health
– PATH
  • Influenza
  • Malaria
  • Enteric disease
– Wellcome Trust
  • Unmet medical need
Primary Funding Opportunities

- Broad spectrum technologies (with potential commercial application)
- Significant United States Government interest in the following areas:
  - Biodefense (it’s not what you imagine!)
  - Emerging Infectious Disease
  - Global Public Health
- CNS - Traumatic Brain Injury/Post-Traumatic Stress/Alzheimer’s
- Rare/Orphan Disease
- Hard Sells – Any Potential Blockbuster
  - Oncology/Cardio/Pain
“Biodefense” Targets

Food- and Waterborne Pathogens
- Diarrheagenic E.coli
- Shigella species
- Salmonella
- Listeria monocytogenes
- Campylobacter jejuni

Antimicrobial resistance

Research on mechanisms of antimicrobial resistance, spread of antimicrobial resistance genes within populations

Modification of existing antimicrobials to overcome emergent resistance

Yellow fever
- Tuberculosis, including drug-resistant TB
- Influenza
- Rabies
- Prions
- Chikungunya virus
- SARS
- Innate immunity
- West Nile Virus
- LaCrosse
- California encephalitis
- VEE
- EEE
- WEE
- Japanese Encephalitis Virus
What Else is Happening in the USG?

- Presidential Combating Antimicrobial Resistance Initiative
- Affordable Care Act (ACA) Implementation
- Legislation and Policy
  - Funding shifts
  - Policy shifts
    - 21st Century Cures
    - Brooks Bill – proposed expansion of the PRV
    - BARDA expanding beyond biodefense and influenza
    - Animal health impact on human health
- Watching the experience of the international health community (learning from them?)
  - Ebola lessons learned/not learned
21st Century Cures

Antibiotic development also features prominently in the *Cures Act*
- Title II,Subtitle G—"Antibiotics Drug Development"—is closely modeled off a previous version of the *Cures Act* and another piece of legislation, the *Promise for Antibiotics and Therapeutics for Health (PATH) Act*.

The bill calls for the creation of a "limited population pathway" for antibacterial and antifungal drugs.
- The pathway would allow a sponsor of a new drug to seek approval for the product intended to treat "a serious or life-threatening disease, condition or indication" that is currently not adequately served by existing therapies.
- The pathway could only be used if the sponsor could identify a specific population in which the medical product would be used.
- Each drug product approved under this pathway would need to be labeled with the following statement: "This drug is indicated for use in a limited and specific population of patients."
- The pathway also provides for the clearance of antimicrobial susceptibility testing devices, which would be used to determine if a particular microorganism is susceptible to a particular drug.

FDA is also required to set up a website to provide recommendations on which bacteria/fungi are susceptible to specific drugs.
A Walk Through Relevant HHS Slides

Drug Development is Expensive, Lengthy, & Risky

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

INH
BARDA ARD
Project BioShield ($5.6B)

PRODUCT PIPELINE

Valley of Death

PROBABILITY OF SUCCESS TO LICENSURE
- 1-3%
- 5-17%
- 10-25%
- 18-35%
- 45-70%
- 90%

TIME
- 3-7 yr
- 0.5-2 yr
- 1-2 yr
- 2-3.5 yr
- 2.5-4 yr
- 1-2 yrs

PIPELINE PHASE COST
- $100M - $130M
- $60M - $70M
- $70M - $100M
- $130M - $160M
- $190M - $220M
- $18M - $20M

A Walk Through Relevant HHS Slides

BARDA Created a Robust & Productive
MCM Development Pipeline

• More than 150 MCM product candidates in development since 2004

A Walk Through Relevant HHS Slides

Project BioShield

- Had early difficulties – but has transformed into a successful program
- Explicit goal of the program – acting as a market guarantee or AMC for developers of products for which there are no commercial markets
- Success of PBS led to PAHPA reauthorization in March 2013
- BARDA expects to procure 8 - 12 new products for the SNS between FY2014-18

Expenditures FY2004-13

A Walk Through Relevant HHS Slides

Project BioShield – Future Funding

• The impact of a continuing resolution will mean that we receive the same level of funding provided in FY 2014
  – FY2015 Request - $408M
  – Received $255M in FY 2014
  – Could have a negative impact on our ability to transition programs to late stage development and procurement and increase burden on our ARD budget for projects that cannot transition

• Anticipated acquisitions, FY2014 – 2018
  – Biodosimetry devices – both POC and HT
  – Thermal burn treatments
  – ARS drugs and therapies
  – Broad Spectrum Antimicrobials
  – Next generation vaccines
  – Chemical antidotes

## FY 2014-18 ARD Investment Priorities

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<tr>
<th>Program</th>
<th>Investments</th>
<th>Comments/Gaps</th>
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| Broad Spectral Antimicrobial | Slight increase | • Supporting WH initiative on antimicrobial resistance  
                           |                            | • Addressing public health threat.                                       |
| Ebola and Marburg        | Increase      | • Currently one program  
                           |                            | • Need funding to support additional programs  
                           |                            | • Cost/burden sharing with our PHEMCE partners.                         |
| Anthrax Vx and Tx        | Maintain      | • Stockpile established  
                           |                            | • Looking for transformative improvements in anthrax vaccines.         |
| Smallpox Vx and Tx       | Maintain      | • Stockpile established  
                           |                            | • Supporting approval of products  
                           |                            | • Transitioning to a more cost effective vaccine.                      |
| Chemical                 | Maintain      | • Enhancing stockpile (CHEMPACKS)  
                           |                            | • Limited pipeline  
                           |                            | • Animal models lacking.                                              |
| Radiation and Nuclear    | Refocus       | • Stockpiling for H-ARS  
                           |                            | • Transitioning towards a pipeline of advanced stage candidates  
                           |                            | • Limited pipeline (Lung, GI, Skin)  
                           |                            | • Developing animal models for GI and Lung.                           |
| Burn and Blood           | Maintain      | • Working with the ABA to develop products with desired characteristics  
                           |                            | • Animal models are difficult  
                           |                            | • Approval pathways unclear.                                          |
What does this all mean to a for-profit biotech?

- There is A LOT of money out there
- USG Partnerships are valuable – BUT have to be managed carefully
- Commercial goals are VERY important to the funders and MUST be the top priority for the company
- “It takes a village”
- Avoid the gold-rush mentality – do your homework!
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