Time Efficiencies to Be Gained from Platform Approaches to Drug Development

Monday, July 27th, 2015

Room 100
National Academy of Sciences Keck Center
500 5th Street NW, Washington DC 20001

Meeting Description

An ad hoc committee will organize a workshop to discuss efficiencies that could be gained by using "platform" approaches in the development of vaccines and therapeutics to protect military personnel and civilians against pathogens. Specifically, the discussion will center on the concept of time efficiencies that can be gained in selected steps of countermeasure development, such as target discovery, production, quality control, and the evaluation of safety and efficacy. The meeting will describe the different types and applications of platforms as applied to product development and how platforms can specifically meet DOD's needs. The committee and the attendees, all coming from various backgrounds, will then review and discuss historical and recent examples of approved platform-based products. The objective is to explore precedents for the approval of platform-based products and to understand where efficiency was gained. Specifically for the DOD's needs, participants will explore the lessons learned and how such lessons enable developers and regulatory agencies to jointly explore innovative and alternative ways to optimize time spent from development to approval and efficiently prepare for the unknown or respond to emergencies. Finally, a forward-looking discussion about the scientific promise of new technologies on the horizon will stimulate discussion on the potential of platform-based approaches for future applications.

Agenda

8:30am **Introduction - Meeting Objectives and Organization**
C. Rick Lyons, Director, Infectious Disease Research Center, Colorado State University[1]

Session 1: DOD-Specific Needs and Current Federal Efforts

Moderator: C. Rick Lyons

8:40 **What Are DOD's Needs and Strategies? What is DOD Currently Developing?**
CDR Franca Jones, Director of Medical Programs, Office of the Assistant Secretary of Defense for Nuclear, Chemical and Biological Defense Programs, DOD

9:00 **BARDA's Perspective and Strategy Regarding Platform Technologies**
Richard Hatchett, Chief Medical Officer and Deputy Director for Strategic Sciences, Biomedical Advanced Research and Development Authority (BARDA), HHS

9:15 **Q&A Session**

Session 2: Background: Present and Future of Platform Technologies

Moderator: Kendall Hoyt, Assistant Professor of Medicine, The Geisel School of Medicine at Dartmouth[1]

9:25 **Terminologies Behind the Word "Platforms"**
John Grabenstein, Executive Director for Medical Affairs and Policy, Merck Vaccines[1]

9:40 **Scientific Promises of New Platform Technologies – Game-Changing Technologies that Introduce Efficiencies**
Andrew Hessel, Distinguished Researcher with Autodesk Inc.'s Bio/Nano Programmable Matter Group

10:00 **Q&A Session**

10:10 **Break**

10:20 **Analytical and Manufacturing Strategies around Platform Development**
Anthony Lubiniecki, Senior Scientific Director, Janssen R&D, LLC[1]

10:35 **Streamline Review of Platform-Based Therapeutics**
Steven Kozlowski, Director, Office of Biotechnology Products, Center for Drug Evaluation and Research, U.S. Food and Drug Administration

10:50 **Q&A session**

[1] Committee Member

Session 3: Efficiencies of Using Platform Technologies to Develop Therapeutics

During this session the participants will tell us about the commonalities among therapeutics that streamline development, review, and production. They will describe challenges and efficiencies gained via repetition or accumulation of experience and define the platform- -ness of their approach. Participants will also discuss promises and limitations of approaching therapeutics development with an emphasis on platform strategies, and the impact of pathogens or molecular targets on those promises and limitations.

VACCINES

Moderator: John Grabenstein

11:00 **Efficiencies Gained by Adapting the Influenza Vaccine with Each Year's Circulating Strain**

Medimmune has an established production process and an approved live-attenuated influenza vaccine that can be adapted each year for the circulating strains.

Speaker: Michael McCarthy, Director and Head, Vaccine Platform Group, MedImmune

11:15 **Efficiencies Gained by Implementing Synthetic Influenza Vaccine Viruses as a Platform Technology for Rapid Response**

Novartis has developed an improved platform approach which reduces the potential time of recovery and production from months to weeks. For instance, the team was able to generate a recombinant influenza virus from new HA and NA sequences within 5 days.

Speaker: Philip R. Dormitzer, Head of U.S. Research, Global Head of Virology, Vice President, Novartis Vaccines and Diagnostics

11:30 **Efficiencies Gained and Not Gained by Adding New Serotypes to Existing Vaccine: Encapsulated Bacterial *Meningococcal* and *Pneumococcal Vaccines***

Pfizer expanded the heptavalent conjugate pneumococcal vaccine (PCV7), developed by Wyeth in 2000, to include protection against 13 strains in 2010 (PCV13; brand name Prevnar 13). PCV13 protects against the bacterial strains responsible for the most severe childhood pneumococcal infections.

Speaker: Wendy Watson, Senior Director, Vaccine Clinical Research, Pfizer, Inc.

11:45 **Efficiencies Gained by Developing Ebola Vaccine Using a Nanoparticle Platform**

Novavax is developing a recombinant viral vaccine against Ebola, using their glycoprotein nanoparticle platform technologies plus proprietary matrix M adjuvant. This Ebola vaccine was similar to a previously developed vaccine against H7N9 influenza. This Ebola vaccine is currently in Phase 1 trials (safety screening in humans) in Australia.

Speaker: Timothy J. Hahn, Sr. V.P., Global Manufacturing Operations, Novavax, Inc.

12:00 **Lunch**

12:45 **Panel Discussion**

- Speakers from above
- *Jeffrey B. Ulmer, Global Head, External Research Head, GSK Vaccines*
- *LTC Victor A. Suarez, Medical Countermeasure Systems - Joint Vaccine Acquisition Program, Joint Product Manager, DOD*
- *Peter Marks, Deputy Director, Center for Biologics Evaluation and Research, U.S. Food and Drug Administration*
- *Norman Baylor, President and CEO, Biologics Consulting Group, Inc.*

MONOCLONAL ANTIBODIES

Moderator: Anthony Lubiniecki

1:45 **Efficiencies Gained by Developing Ebola Virus Disease Treatment Using Chimeric Monoclonal Antibodies as a Platform**

Mapp Biopharmaceutical Inc is developing ZMapp™, an experimental three chimeric monoclonal antibody (mAb) treatment for Ebola virus disease. The clinical trial was launched in February in Liberia and the U.S. and later was expanded to Sierra Leone and soon to Guinea.

Speaker: Kenneth G. Payie, Vice President, Product Development, Mapp Biopharmaceutical Inc.

2:00 **Efficiencies Gained by Developing New Treatments Using Adimab Platform for Antibody Discovery and Optimization**

Adimab's technology is designed to accelerate development timelines from target to fully human therapeutic antibodies.

Speaker: Tillman U. Gerngross, CEO, Adimab, LLC

2:15 **Efficiencies Gained by Using Innovative Platform Drug Development to Facilitate Approval under Breakthrough Designation**

FDA's Breakthrough Therapy designation is a mean of reducing the amount clinical information required to move from Phase I into late-phase registration trials, based on a strong initial signal of efficacy. The talk will cover how Chemistry, Manufacturing, and Controls (CMC) platform technologies can integrate with the breakthrough status.

Speaker: E. Morrey Atkinson, Vice President, Biologics Development, Bristol-Myers Squibb

2:30 **Panel Discussion**

- Speakers from above
- *LTC Eric G. Midboe, Joint Product Manager, BioDefense Therapeutics, DOD*
- *David Frucht, Director, Division of Biotechnology Review and Research II, Center for Drug Evaluation and Research, U.S. Food and Drug Administration*

3:15 **Break**

RNA THERAPEUTICS

Moderator: C. Rick Lyons

3:30 **Challenges and Efficiencies Gained by Developing an Ebola Therapeutic Based on RNA Interference and Lipid Nanoparticle Platform Technologies**

Tekmira first developed TKM-Ebola and then TKM-Ebola-Guinea, two anti-Ebola viral RNAi therapeutics utilizing Tekmira's Lipid Nanoparticle Platform (LNP) technology. Tekmira commenced a Phase II clinical trial evaluating TKM-Ebola-Guinea in infected patients in Sierra Leone. Another product, TKM-Marburg will be described to highlight the company's platform technology.

Speaker: Lloyd Jeffs, Director, TKM-Ebola Program, Tekmira Pharmaceuticals Corporation

3:45 **Efficiencies Gained by Using an RNA Platform to Develop a Potential Ebola Treatment**

Phosphorodiamidate morpholino oligomers (PMOs) are uncharged nucleic acid-like molecules designed to inactivate the expression of specific genes. The PMO chemistry platform has been adapted to diverse therapeutics application.

Speaker: Patrick Iversen, Professor, Oregon State University, Corvallis

4:00 **Panel Discussion**

- Speakers from above
- *LTC Eric G. Midboe, Joint Product Manager, BioDefense Therapeutics, DOD*
- *Edward Cox, Director, Office of Antimicrobial Products, Center for Drug Evaluation and Research, U.S. Food and Drug Administration*

Session 4: Structured Discussion

Moderator: John Grabenstein

4:30 **Discussion:** (Fill the Matrix below with what we've heard)

	Target Discovery	**GMP Production**	**Quality Control** (CMC Activity, Storing, Shipping, Stabilizing)	**Safety**	**Efficacy**
VACCINES					
MedImmune					
Novartis					
Pfizer					
Novavax					
MONOCLONAL ANTIBODIES					
Mapp Biopharmaceutical					
Adimab					
Bristol-Myers Squibb					
RNA THERAPEUTICS					
Tekmira Pharmaceuticals					
Oregon State University					

5:15 **Adjourn**

Committee on Support to the Department of Defense's Programs to Counter Biological Threats

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CLOSED SESSION: COMMITTEE AND STAFF ONLY

8:30am	**Discussion of Day 1 Meeting and Next Meeting Topics**

OPEN SESSION

9:30am	**Meeting Discussion between Committee and Sponsor** • Sponsor Thoughts • Meeting Insights from Committee Members • Summary of Meeting Insights
10:30	**Updates on Impacts of Previous Meetings** *CDR Franca Jones*
10:45	**Break**
11:00	**Beyond the current contract**
12:15	**Lunch**
1:00	**Adjourn**