

# Identification and Use of Biomarkers to Advance Development of Preventive Vaccines

**September 16 & 17, 2019**

5601 Fishers Lane, Rm. 1D13  
Rockville, MD 20852

## **Meeting goals:**

Recent legislation, including the 21st Century Cures Act and Prescription Drug User Fee Act (PDUFA) VI, encourages use of biomarkers to enhance development and approval of new and innovative drug and biological products. Particularly in the field of vaccines to prevent infectious disease, a well-characterized biomarker has tremendous potential value, because it can enhance basic research, facilitate vaccine development, and guide the effective use of vaccines. To facilitate the realization of this potential, FDA's Center for Biologics Evaluation and Research (CBER), NIH's National Institute for Allergy and Infectious Diseases (NIAID), and the Coalition for Epidemic Preparedness Innovations (CEPI) are partnering to convene this workshop. The purpose is to exchange information with stakeholders from industry, academia, and government about the scientific, clinical, and regulatory challenges encountered in the discovery, characterization, and qualification of biomarkers for preventive vaccines for infectious diseases indications. The objectives of the workshop include:

- Provide the context and understand the importance of biomarkers in vaccine discovery and development, including through review of successful case examples.
- Clarify the regulatory framework that informs the use of biomarkers in vaccine development and licensure.
- Assess the quality of the evidence for biomarkers to support decisions (regulatory, programmatic, and otherwise) regarding candidate and licensed vaccine products for specific infectious diseases.
- Explore how new technologies and innovations can be applied to advance the science of vaccine-associated biomarkers.
- Understand the institutional perspectives/priorities with respect to the use of biomarkers for vaccine development and deployment across a wide range of stakeholders.

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## DRAFT AGENDA

### DAY 1

#### INTRODUCTION

8:30 – 8:40

#### **Welcome**

Marion Gruber, PhD  
Director, Office of Vaccines Research and Review (OVRR), CBER/FDA

8:40 – 8:50

#### **Successes and opportunities using biomarkers to advance basic and clinical science for infectious diseases and vaccine discovery**

Anthony Fauci, MD  
Director, National Institute of Allergy and Infectious Diseases  
NIH

8:50 – 9:10

#### **The importance of biomarkers in the development of vaccines against diseases with epidemic potential**

Debra Yeskey, PharmD  
Head of Regulatory Affairs, North America  
CEPI

9:10 – 9:40

#### **Use of biomarkers for regulatory decision-making in vaccine development and licensure application review**

Jeff Roberts, MD  
Associate Director for Medical Countermeasures and Scientific Affairs,  
OVRR/CBER/FDA

9:40 – 10:10

#### **New technologies and computational capacities and the future of vaccine biomarker development**

Barney Graham, MD, PhD  
Deputy Director, Vaccine Research Center  
Chief, Viral Pathogenesis Laboratory and Translational Science Core  
Viral Pathogenesis Laboratory

*And*

Dean Follmann, PhD  
Chief, Biostatistics Research Branch  
NIAID/NIH

10:10 – 10:30

Break

#### **SESSION 1: Highlights from selected case examples: lessons learned and next steps**

10:30 – 10:50

#### **Mechanistic approaches to developing biomarkers for Zika vaccine development**

Theodore Pierson, PhD,  
Chief, Viral Pathogenesis Section  
Chief, Laboratory of Viral Diseases  
Laboratory of Viral Disease, NIAID

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- 10:50 – 11:10      **Clinical trials of Zika vaccine candidates: pros and cons of different biomarker endpoints**  
Julie Ledgerwood, D.O.  
Chief Medical Officer and  
Chief, Clinical Trials Program  
Vaccine Research Center  
NIAID, NIH
- 11:10 – 11:30      **Evidence from animal studies for an antibody-based biomarker to support effectiveness of chikungunya vaccines**  
Katrin Ramsauer, PhD  
Chief Scientific Officer, Themis BioScience GmbH
- 11:30 – 11:50      **Endpoints for prophylactic congenital CMV vaccine development**  
Kevin Russell, MD, MTM&H  
Section Head, Clinical Research Vaccines  
Merck Research Laboratories  
*And*  
Long Wang, MD, PhD  
Director, Global Regulatory Team Leader, Vaccines & Infectious Disease  
Merck Inc.
- 11:50 – 12:15      **Q&A and discussion**
- 12:15 – 1:15        **Lunch**

### **SESSION 2: Progress on the development of biomarkers in animal models of hemorrhagic fever viruses**

- 1:15 – 1:35        **Use of animal modeling to develop an antibody-based biomarker to support effectiveness of Ebola vaccines**  
Nancy Sullivan, PhD  
Chief, Biodefense Research Section  
Viral Pathogenesis Laboratory, NIAID
- 1:35 – 1:55        **Candidate biomarkers to support clinical development of Ad26.ZEBOV and MVA-BN-Filo vaccine**  
Jenny Hendricks, PhD  
Head Biomarkers, Viral Vaccines, Janssen Vaccines, ID&V
- 1:55 – 2:15        **Development of quadrivalent Filovirus/Lassa vaccine and considerations for use of biomarkers**  
Rong Xu, MD, PhD  
Director of Immunology, Profectus Biosciences, Inc.
- 2:15 – 2:35        **Comparisons of naturally acquired immune response vs vaccine induced immune responses to Ebola**  
Professor Miles W. Carroll  
Deputy Director, Head of Research & Development Institute

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National Infections Service, Public Health England

2:35 – 3:00 **Q&A and discussion**

3:00 – 3:15 **Break**

### **SESSION 3: Updates on selected topics**

3:15 – 3:35 **Prospects for identifying correlates of protection in clinical studies of HIV monoclonal antibody candidates**

John Mascola, MD

Director, Vaccine Research Center, NIAID

3:35 – 3:55 **Use of biomarkers to support an indication for the Anthrax postexposure prophylaxis (PEP)**

Josh Reese, PhD

Senior Director, Vaccine Research & Development, Vaccines & Anti-Infectives Business Unit, Emergent BioSolutions

3:55 – 4:15 **Next generation influenza vaccines: Recent activities in identifying correlates of protection and biomarkers for next generation influenza vaccines**

Raffael Nachbagauer, MD, PhD

Assistant Professor, Department of Microbiology Icahn School of Medicine at Mount Sinai

4:15 – 4:45 **Q&A and discussion**

4:45 **Adjourn**

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## DAY 2

### **SESSION 4: Regulatory Considerations – the potential role for the Biomarker Qualification Program (BQP)**

- 9:00 – 9:10            **Welcome back and overview of the day’s agenda**
- 9:10 – 9:35            **Use of Drug Development Tools Biomarker Qualification Program to advance development and licensure of new vaccines**  
Sarah K. Browne, MD  
Senior Advisor-Clinical, Division of Vaccines and Related Product Applications, OVRP/FDA/CBER
- 9:35 – 10:00        **FDA qualification of *P. falciparum* 18s rRNA/DNA: lessons learned from qualification of a biomarker for a specific Context of Use (COU)**  
Sean Murphy, MD, PhD  
Associate Professor, Laboratory Medicine, University of Washington Medical Center
- 10:00 – 10:20        **Q&A and discussion**
- 10:20 – 10:40        **Break**

### **SESSION 5: Practical Considerations**

- 10:40 – 11:05        **Using systems biology and “omics” to search for biomarker signatures**  
Nathalie Garçon, PhD, Chief Executive and Scientific Officer, Bioaster
- 11:05 – 11:30        **Overview of design and implementations of CDC’s study to assess candidate biomarkers for GBS vaccine development**  
Barbara Mahon, MD, MPH  
Director, Division of Bacterial Diseases  
National Center for Immunization and Respiratory Diseases (CDC)
- 11:30 – 11:55        **Development of assays for use in vaccines intended for maternal immunization to prevent neonatal group B strep infection**  
Kirsty Mehring Le Doare, PhD  
Professor, Paediatric Infectious Diseases Research Group  
St George’s, University of London
- 11:55 – 12:15 pm    **Q&A and discussion**
- 12:15 – 1:15            **Lunch**

**Session 6: Stakeholder perspectives on current and future uses of biomarkers in vaccine development, licensure, and post-licensure surveillance**

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1:15 – 2:05

### **Brief summary of institutional perspective (10 minutes each)**

**Phyllis Arthur**, Vice President, Infectious Diseases & Diagnostics Policy at Biotechnology Innovation Organization (BIO)

**David Kaufman**, Chief Medical Officer, Bill & Melinda Gates Medical Research Institute

**David Kaslow**, Vice President, Essential Medicines Director  
PATH

**Marco Cavaleri**, Head of Office, Anti-infectives and Vaccines in the Human Medicines Evaluation Division

**Gary Disbrow**, Director, Division of CBRN Countermeasures at US Department of Health and Human Services, BARDA

2:05 – 3:00

### **Panel Discussion with stakeholders**

3:00 – 3:10

### **Wrap-up**

3:10 – 3:15

### **Closing Comments**