FDA/NIH/CEPI BIOMARKERS WORKSHOP

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 – 8:50 **Welcome**
Marion Gruber, PhD
Director, Office of Vaccines Research and Review (OVRR), CBER/FDA

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

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

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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**
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
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 Reece, 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**

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, OVRR/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**

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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

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**