A workshop co-hosted by:

INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

CMTP
Center for Medical Technology Policy

Board on Health Sciences Policy
Roundtable on Translating Genomic-Based Research for Health

Evidence for Clinical Utility of Molecular Diagnostics in Oncology: A Workshop

May 24, 2012

PEW DC Conference Center
901 E. St., N.W.
Washington, DC, 20004

Workshop Objectives:

- To assess the evidentiary requirements for clinical validity and clinical utility of molecular diagnostics which are used to guide treatment decisions for cancer patients.
- To discuss methodologies including innovative models related to demonstrating these evidentiary requirements that meet the needs of all stakeholders.
- To consider innovative, sustainable research collaborations for generating evidence of clinical utility which involve multiple stakeholders.

8:30 A.M. **Welcoming Remarks and Charge to Workhop Speakers and Participants**

Robert McCormack, *Workshop Co-Chair*
Head of Technology Innovation and Strategy
Veridex, LLC

8:50 A.M. **Stakeholder-Informed Methods for Evaluating Clinical Utility**

Sean Tunis
Director
Center for Medical Technology Policy

SESSION I: EVIDENCE UTILIZATION

Session Focuses: **Stakeholder requirements for and evaluation of evidence**. **Identifying needs, gaps, and issues in guideline development processes, payer coverage policy, provider and patient decision-making.**

Moderator: Elizabeth Mansfield
Director of the Personalized Medicine Staff
Office of In Vitro Diagnostic Device Evaluation and Safety
Center for Devices and Radiological Health
Food and Drug Administration

9:05 – 11:05 A.M. (15 minute talks by speakers)

Guideline Development

Gary H. Lyman
Professor of Medicine; Director, Comparative Effectiveness and Outcomes Research – Oncology
Duke University School of Medicine and the Duke Cancer Institute

Al B. Benson III
Professor of Medicine
Associate Director for Clinical Investigations
Robert H. Lurie Comprehensive Cancer Center, Northwestern University

Payer Perspectives

Elaine Jeter
Medical Director
Palmetto GBA

Lee Newcomer
Senior Vice President, Oncology
United HealthCare Corporation

10:05 A.M. BREAK (15 minutes)

Provider Perspective

Lloyd Everson
Vice Chairman and Founder
The US Oncology Network

Academic Health System Perspective

Robert Bast
Vice President for Translational Research; Internist and Professor of Medicine, Department of Experimental Therapeutics, Division of Cancer Medicine; Harry Carothers Wiess Distinguished University Chair for Cancer Research, The University of Texas MD Anderson Cancer Center

Patient Perspective

Deborah E. Collyar
President
Patient Advocates in Research

11:05 – 11:50 A.M. **Discussion with speakers and participants**

11:50 A.M. LUNCH

SESSION II: STUDY DESIGN AND ANALYSIS

Session Focuses: Methodologies for evidence generation and synthesis. Statistical approaches for study design and analysis.

Moderator: Patricia Deverka
Senior Research Director
Center for Medical Technology Policy

12:35 P.M. - 2:15 P.M. (20 minute talks by speakers)

Evolution of Translational Omics: Lessons Learned and the Path Forward

Debra Leonard
Professor and Vice Chair, Department of Pathology and Laboratory Medicine
Director of the Clinical Laboratories
Weill Cornell Medical Center

Comparative Effectiveness Research Methodologies for Generating and Synthesizing Evidence for Cancer Genomics

Andrew N. Freedman
Chief, Clinical and Translational Epidemiology Branch
Epidemiology and Genomics Research Program
Division of Cancer Control and Population Sciences
National Cancer Institute

Designing Studies to Evaluate Biomarkers for Clinical Applications

Lisa M. McShane
Mathematical Statistician
Biometric Research Branch
Division of Cancer Treatment and Diagnosis
National Cancer Institute

Assessing Cost Effectiveness for Oncology-based Molecular Diagnostics

Kathryn Phillips
Professor of Health Economics and Health Services Research
Director and Founder, UCSF Center for Translational and Policy Research on Personalized Medicine
Dept. of Clinical Pharmacy/School of Pharmacy, UCSF
Institute for Health Policy Studies, and UCSF Comprehensive Cancer Center
University of California, San Francisco

Advancing the Utility of Oncology Diagnostics

Noel Doheny
Chief Executive Officer
Epigenomics Inc.

2:15 – 3:00 P.M. **Discussion with speakers and participants**

3:00 P.M. BREAK (15 minutes)

SESSION III: ADVANCING MOLECULAR DIAGNOSTICS FOR ONCOLOGY

Session Focus: A discussion on pathways forward and next steps – Partnerships to accelerate evidence development.

Moderator: Margaret Piper
Director of Genomic Resources
Blue Cross Blue Shield Association

3:15 P.M. – 4:30 P.M. (15 minute talks by speakers)

Biomarker Studies in Multi-center Cancer Clinical Trials: The Role of Cooperative Groups

Richard Schilsky
Professor of Medicine
Chief, Section of Hematology-Oncology
Deputy Director, Comprehensive Cancer Center
University of Chicago

Partnering for the Cure: An Innovative Role for Academia in Oncology Drug and Diagnostic Development

Howard I. Scher
D. Wayne Calloway Chair in Urologic Oncology
Sidney Kimmel Center for Prostate and Urologic Cancers
Chief, Genitourinary Oncology Service
Memorial Sloan-Kettering Cancer Center

Patient Approaches to Generating Evidence

Deborah E. Collyar
President
Patient Advocates in Research

Novel Partnership Strategies for Using Outcomes Data to Develop Clinical Utility Evidence

Gabriela Lavezzari
Director of Development, Diagnostics
Express Scripts

Assessing Clinical Utility with Real-World Evidence

Greg Rossi
Vice President, Payer & Real World Evidence
Astrazeneca UK

4:30 – 5:15 P.M. **Discussion with speakers and participants on paths forward**

SESSION IV: FINAL REMARKS

5:15 P.M. **Concluding Remarks**

Robert McCormack, *Workshop Co-Chair*
Head of Technology Innovation and Strategy
Veridex, LLC

5:30 P.M. **Adjourn**