FDA-AACR Real-world Evidence Workshop

July 19, 2019

Bethesda Doubletree by Hilton | Bethesda, MD

Workshop Cochairs:

U.S. Food and Drug Administration:

Sean Khozin, MD, MPH, Associate Director, Oncology Center of Excellence, Director, Information Exchange and Data Transformation (INFORMED), U.S. Food and Drug Administration

Pallavi Mishra-Kalyani, PhD, Team Leader, Division of Biometrics V, Office of Biostatistics, Center for Drug Evaluation and Research, U.S. Food and Drug Administration

American Association for Cancer Research:

Deborah Schrag, MD, MPH, Chief, Division of Population Sciences, Dana-Farber Cancer Institute

AGENDA

INTRODUCTION

8:00 AM **Introduction & Objectives**
Pallavi Mishra-Kalyani, PhD, U.S. Food and Drug Administration

SESSION I: INTRO TO REAL-WORLD EVIDENCE

This session will introduce real-world evidence concepts and the utility of using real-world data sources.

8:05 AM **Keynote: FDA Framework on Real-world Evidence**
Jacqueline Corrigan-Curay, JD, MD, U.S. Food and Drug Administration

8:45 AM **Real-world evidence: Utility and clinical decision support**
Elad Sharon, MD, MPH, National Cancer Institute

SESSION II: PREMARKET USE CASES
SESSION MODERATOR: PALLAVI MISHRA-KALYANI, PHD

This session will provide examples of using real-world evidence in drug development.

9:05 AM **Use of real-world evidence to assess the benefits of new therapies in regulatory decision-making: a case study of historical data for adults with r/r acute leukemia (ALL).**
Michael A. Kelsh, PhD, MPH, Amgen

9:25 AM **Using a comparative effectiveness study in support of regulatory and payer submissions – an example from 1L TNBC**
William Capra, PhD, Genentech

9:45 AM **Use of real-world evidence to inform clinical development**
Weili He, PhD, AbbVie

10:05 AM **PANEL DISCUSSION and AUDIENCE Q&A**

Panelists: **Session II speakers and the following additional panelists:**
Laleh Amiri-Kordestani, MD, U.S. Food and Drug Administration
Rajeshwari Sridhara, PhD, U.S. Food and Drug Administration
Cynthia Huang, MD, MBA, Merck

10:30 AM **BREAK**

SESSION III: POSTMARKET USE CASES
SESSION MODERATOR: PALLAVI MISHRA-KALYANI, PHD

This session will provide examples of using real-world evidence in postmarket situations.

10:50 AM **Postmarket real-world data perspectives: Oncology registration use cases**
Albert L. Kraus, PhD, Pfizer

11:10 AM **Case study illustrating that a synthetic control arm derived from historical clinical trials and matching of baseline characteristics can replicate the overall survival of a randomized control arm**
Ruthanna Davi, PhD, Acorn AI

11:30 AM **RWE Pilot Project Data: Characterization of real-world endpoints to assess long-term benefit**
Jeff Allen, PhD, Friends of Cancer Research

11:50 AM **PANEL DISCUSSION and AUDIENCE Q&A**

Panelists: **Session III speakers and the following additional panelists:**
Laleh Amiri-Kordestani, MD, U.S. Food and Drug Administration
Mark Levenson, PhD, U.S. Food and Drug Administration
Joohee Sul, MD, U.S. Food and Drug Administration
Frank W. Rockhold, PhD, Duke Clinical Research Institute

12:15 PM **LUNCH (ON YOUR OWN)**

SESSION IV: LARGE GENOMIC DATABASES & REAL-WORLD EVIDENCE
SESSION MODERATOR: DEBORAH SCHRAG, MD, MPH

This session will explore large genomic databases and digital data as real-world sources of information.

1:15 PM **CancerLinQ and RWE: Accomplishments, barriers to opportunity, and a path toward shared success**
Wendy Rubinstein, MD, PhD, CancerLinQ

1:27 PM **The NCI Genomic Data Commons and Cancer Research Data Commons and real-world data**
Robert Grossman, PhD, University of Chicago

1:39 PM **Moving from reactive to proactive medicine through data science**
William S. Dalton, PhD, MD, M2Gen

1:51 PM **A real-world clinico-genomic data platform to accelerate research and development**
Neal J. Meropol, MD, Flatiron Health

2:03 PM **Real-world evidence at the bedside: Two use cases**
Gary Palmer, MD, JD, MBA, MPH, Tempus

2:15 PM	**Utilizing a real-world clinical+molecular data platform to advance clinical care and outcomes research** **Jonathan Hirsch,** Syapse
2:27 PM	**Integrating genomics and phenomics: AACR Project GENIE** **Deborah Schrag, MD, MPH,** Dana-Farber Cancer Institute
2:39 PM	**PANEL DISCUSSION and AUDIENCE Q&A**
Panelists:	**Session IV speakers and the following additional panelist:** **Gideon M. Blumenthal, MD,** U.S. Food and Drug Administration
3:05 PM	**BREAK**

SESSION V: REAL-WORLD EVIDENCE — FUTURE DIRECTIONS
SESSION MODERATOR: ANDREA CORAVOS

This session will explore the future of real-world evidence.

3:20 PM	**Algorithms, connected technologies, and generating real-world evidence** **Andrea Coravos,** Elektra Labs
3:35 PM	**Intelligent Health (iHealth), an intramural NCI digital health initiative** **James Gulley, MD, PhD,** National Cancer Institute
3:50 PM	**Perpetual trials for the generation of RWD in precision oncology** **Mark Shapiro, MBA, PhD,** xCures
4:05 PM	**PANEL DISCUSSION and AUDIENCE Q&A**
Panelists:	**Session V speakers and the following additional panelists:** **Steven J. Lemery, MD, MHS**, U.S. Food and Drug Administration **Pallavi Mishra-Kalyani, PhD,** U.S. Food and Drug Administration **Oliver Bogler, PhD,** ECHO Institute **Rohit Borker, PhD,** Novartis **Deborah Schrag, MD, MPH,** Dana-Farber Cancer Institute

CONCLUSION

4:55 PM	**Concluding remarks** **Deborah Schrag, MD, MPH,** Dana-Farber Cancer Institute
5:00 PM	**ADJOURN**