DIA Companion Diagnostics Conference 2015: Connecting Science and Regulation to Advance Innovation in Precision Medicine and Companion Diagnostics

September 30 – October 1

Hyatt Regency Bethesda | Bethesda, MD

As of September 21, 2015

PROGRAM COMMITTEE:

Miu Chau, PhD
Regulatory Program Director
Genentech, A Member of the Roche Group

Jennifer Dudinak, PharmD
Vice President
Global Regulatory Affairs
GlaxoSmithKline

Jennifer Shen, PhD, RAC
Scientific Reviewer
CDRH/OIR/DMGP, FDA

Eric Slosberg, PhD
Senior Director
Translational Medicine
Novartis Oncology

Jeffrey Stuart, PhD, RAC
Director, Regulatory Affairs
Novartis Pharmaceuticals Corporation

OVERVIEW:

Advances in scientific knowledge and technology are driving growth and innovation for companion diagnostics, thereby enabling personalized therapies that:

- Target those patients most likely to benefit from treatment
- Exclude those patients most likely to suffer harm, and,
- Can be monitored during treatment for clinically significant changes.

Drug and device pairings allow treatment decisions to be tailored for each patient. However, development of companion diagnostics with targeted drug therapy continues to face challenges as regulatory policy tries to keep up with rapid advances in technology and health care demands.

FEATURED TOPICS:

- US and Global Regulations
- Innovative Clinical Trial Designs
- Next Generation Sequencing as Diagnostic Devices
- Laboratory-Developed Tests and Its Impact on Precision Medicine

LEARNING OBJECTIVES:

At the conclusion of this conference , participants should be able to:

- Discuss the global and US regulatory framework for companion diagnostics
- Identify key challenges and potential paths in moving forward for the drug and companion diagnostic manufacturers
- Describe the impact of laboratory-developed tests on precision medicine

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Type of Activity: Knowledge

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- **Day 2, Session 6:** 1.5 contact hours or .15 CEUs, 0286-0000-15-114-L01-P
- **Day 2, Session 8:** 1.0 contact hour or .1 CEU, 0286-0000-15-115-L03-P

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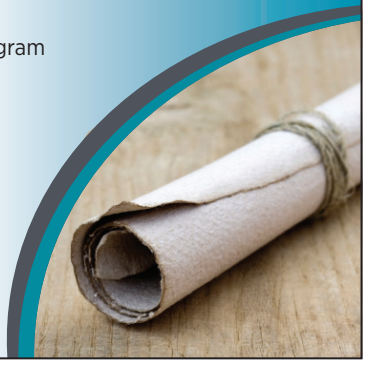

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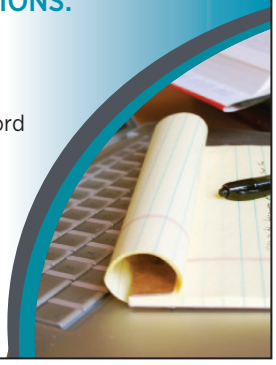

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WEDNESDAY, SEPTEMBER 30

7:15AM-5:00PM **REGISTRATION**

7:15-8:15AM **CONTINENTAL BREAKFAST**

8:15-8:30AM **WELCOME AND OPENING REMARKS**

Jennifer Shen, PhD, RAC
Scientific Reviewer
CDRH/OIR/DMGP, FDA

8:30-10:00AM **SESSION 1: EVOLVING FDA REGULATIONS OF GENETIC TESTS**

SESSION CHAIR:

Jennifer Shen, PhD, RAC
Scientific Reviewer
CDRH/OIR/DMGP, FDA

FDA has co-approved a number of companion diagnostics and targeted therapeutics in the last few years. It has also become apparent that multiple biomarker analyses are increasingly utilized to predict outcomes, select patients for treatment, or provide prognostic values in oncology patient care. This session will focus on regulatory strategies and development considerations when employing a companion diagnostic in a therapeutic development program. Examples will be discussed to illustrate how regulatory policies are developed to keep pace with the fast-evolving field of genomic testing. A case study about the recent co-approvals of BRACAnalysis companion diagnostics and olaparib will be presented, and a panel discussion will follow to share the different perspectives of a successful co-development program.

Office of In Vitro Diagnostics and Radiological Health - Developments

Aaron Schetter, PhD
Scientific Reviewer
CDRH/OIR/DMGP, FDA

Case Study: BRACAnalysis Companion Diagnostics and Olaparib Co-approvals

Maria Orr, PhD
Diagnostic Team Director
AstraZeneca

Gwynn Ison, MD
Medical Officer
CDER/OHOP, FDA

Jolette Franco, MT (ASCP), MBA
Regulatory Affairs Manager
Myriad Genetic Laboratories, Inc

Eunice Lee, PhD
Acting Branch Chief
CDRH/OIR/DMGP, FDA

Reena Philip, PhD
Division Director
CDRH, FDA

Panel Discussion

Case study participants

10:00-10:30AM **REFRESHMENT AND NETWORKING BREAK**

10:30AM-12:00PM SESSION 2: INNOVATIVE CLINICAL TRIAL DESIGNS IN PRECISION MEDICINE

SESSION CHAIR

Eric Slosberg, PhD
Senior Director
Translational Medicine
U.S. Clinical Development and Medical Affairs
Novartis Oncology

The hypercompetitive environment to gain approvals for new drugs (or new indications for already approved drugs) applies pressure onto a slow, cumbersome clinical trial system. This session will discuss novel ways to approach this. The development of new patient (molecular) profiling technologies and companion diagnostics, their incorporation into innovative clinical trial designs, and resultant clinical and regulatory issues and concerns will be addressed. Also the rapid disruption of the oncology landscape by immuno-oncology agents will be discussed.

National Cancer Institute/Consortium Initiatives in Novel Clinical Trial Designs

James Doroshow, MD
Deputy Director
National Cancer Institute

Diagnostic Tests Used Within a Drug Program: Overcoming the Challenges of Codevelopment

Christopher Leptak, MD, PhD
Biomarker and Companion Diagnostic Lead
Office of New Drugs
CDER, FDA

Leveraging Advanced Companion Diagnostics in Innovative Clinical Trial Designs: A Physician's Perspective

Patricia LoRusso, DO
Associate Center Director – Innovative Medicine
Smilow Cancer Center, Yale University

Companion Diagnostics for Immune Checkpoint Therapy

David Rimm, MD, PhD
Professor of Pathology and of Medicine (Medical Oncology)
Director of Pathology Tissue Services
Director of Translational Pathology
Yale University

12:00-1:30PM LUNCHEON AND NETWORKING

1:30-3:00PM SESSION 3: PART ONE: CHALLENGES IN IMPLEMENTING COMPANION DIAGNOSTICS IN THE CLINICAL LABORATORY

SESSION CHAIR

Rosanne Welcher, PhD, MBA, RAC
Senior Director
Quality Assurance
Regulatory and Clinical Affairs
Dako North America, *an Agilent Technologies Company*

Clinical and reference laboratories are essential contributors to the success of companion diagnostics in precision medicine. This session will address some of the challenges these labs face and the impact on precision medicine. Topics will include how laboratory-developed tests play a role in clinical trials and beyond, and how issues like pre-screening may bias the clinical trial results and statistical analysis. FDA and industry experts will provide their perspective on these topics.

Implementing Quality System Regulations

Russel Henderson
Project Manager
Myriad Genetics, Inc.

FDA Quality System Implementation for Companion Diagnostics Laboratory Developed Tests, A Case Study

Joshua Levin, PhD, RAC
Postmarket Team Lead
CDRH/OIR/DMGP, FDA

Statistical Issues with Local Testing and Next Generation Sequencing in Clinical Trials

Douglas Robinson, PhD
Global Head, Biomarkers and Diagnostics Biometrics
Novartis Institute for BioMedical Research Inc.

Issues of Prescreening Bias for Companion Diagnostics

Yuying Jin, PhD
Mathematical Statistician
CDRH/OSB, FDA

3:00-3:30PM REFRESHMENT AND NETWORKING BREAK

3:30-5:00PM **SESSION 4: PART TWO: CHALLENGES IN THE DESIGN AND DEVELOPMENT OF COMPANION DIAGNOSTICS: A ROUNDTABLE DISCUSSION**

SESSION CHAIRS:

Rosanne Welcher, PhD, MBA, RAC
Senior Director
Quality Assurance
Regulatory and Clinical Affairs
Dako North America, *an Agilent Technologies Company*

Miu Chau, PhD
Regulatory Program Director
Genentech, A Member of the Roche Group

Jeffrey Stuart, PhD, RAC
Director
Regulatory Affairs
Novartis Pharmaceuticals Corporation

This will be an interactive session with the audience. Various challenges in clinical trial design and regulatory strategies will be presented to the audience. During group discussions facilitated by moderators, participants will provide their perspective, experience and possible solutions to overcome the challenges.

1. Impact of Local Testing on Clinical Trials for Drug / Companion Diagnostics (Pre-Screening)

MODERATOR:

Douglas Robinson, PhD
Global Head, Biomarkers and Diagnostics Biometrics
Novartis Institute for BioMedical Research Inc.

2. Specific Challenges in Bridging Studies

MODERATOR:

Yuying Jin, PhD
Mathematical Statistician
CDRH/OSB, FDA

3. How to Minimize the Impact to Patient Care from Multiple Companion Diagnostics Assays to the Same Biomarker

MODERATOR:

Reena Philip, PhD
Division Director
CDRH/OIR/DMGP, FDA

4. Challenges for Laboratory-Developed Tests in Post-Market of Approved Companion Diagnostics Assays

MODERATOR:

Joshua Levin, PhD, RAC
Postmarket Team Lead
CDRH/OIR/DMGP, FDA

5:00-6:00PM **NETWORKING RECEPTION**

THURSDAY, OCTOBER 1

7:30AM-12:30PM **REGISTRATION**

7:30-8:30AM **CONTINENTAL BREAKFAST**

8:30-10:30AM **SESSION 5: GLOBAL REGULATORY PERSPECTIVE**

SESSION CHAIR

Miu Chau, PhD
Regulatory Program Director
Genentech, A Member of the Roche Group

Companion diagnostics development needs to take into consideration the global regulatory requirements for worldwide registrations. With the increasing number of drugs requiring companion diagnostics to identify patients for treatment, the global regulatory framework is gradually changing to keep up with the innovations. For example, in the European Commission's proposed new framework, companion diagnostics will be classified as high individual risk or moderate public health risk (Category C) and require conformity assessment by a notified body instead of by self-certification. In Japan, the Pharmaceuticals and Medical Devices Agency (PMDA) published 'A Technical Guidance on Development of In-Vitro Companion Diagnostics and Corresponding Therapeutic Products' in 2013. This session will explore the potential impact of the changing global regulatory framework on the different aspects of companion diagnostics development.

Regulatory Perspective and Challenges Regarding Companion Diagnostics in Japan

Sumimasa Nagai, MD, PhD
Senior Assistant Professor
The University of Tokyo and PMDA
Japan

Overview of Current and Future European Regulatory Requirements for Companion Diagnostics

Shayesteh Fuerst-Ladani, MBA, MsC, Eng
Managing Director and Founder
SFL Regulatory Affairs and Scientific Communication, Switzerland

China Food and Drug Administration Enters Into the Era of Co-Review/Co-Approval of Companion Diagnostics

Rosanne Welcher, PhD, MBA, RAC
Senior Director
Quality Assurance
Regulatory and Clinical Affairs
Dako North America, *an Agilent Technologies Company*

Principles for Codevelopment of an In Vitro Companion Diagnostic Device with a Therapeutic Product

Pamela Bradley, PhD
Staff Fellow
CDRH/OIR, FDA

10:30-11:00AM **REFRESHMENT AND NETWORKING BREAK**

11:00AM-12:30PM SESSION 6: ADVANCING REGULATORY PATHWAYS FOR NEW FRONTIERS IN MOLECULAR TESTING, INCLUDING NEXT-GENERATION SEQUENCING

SESSION CHAIR

Jennifer Dudinak, PharmD
Vice President
Regulatory Affairs, Oncology
GlaxoSmithKline

The speed of evolution of scientific knowledge to identify molecularly-targeted agents and advancements in technology (such as multiple-marker diagnostics) offers great promise in identifying subsets of patients who will likely benefit (or experience minimal/detrimental effect) from novel therapies. Multiple marker diagnostics (multiplex) or next-generation sequencing (NGS) platforms offer the ability to test for a wide array of biomarkers and mutations at once. These platforms can be instrumental in fostering efficient health care utilization, maximizing sampling for tissue/specimen/biopsy and advancing innovations in molecularly-targeted therapeutic development and registration across a variety of therapy areas. There are several regulatory and development strategic considerations when employing a multiple-marker diagnostic approach in a therapeutic development program. This session will explore challenges and opportunities of utilizing a multiple-marker approach, including how stakeholders can partner together to advance the regulatory framework. Pertinent highlights from recent FDA workshops on NGS and precision medicine will also be discussed.

Application of Multi-Analyte Testing in Clinical Development: Practical Considerations

Anne-Marie Martin, PhD
VP, Head of Biomarker Research and Development
Adaptimmune, LLC

Regulatory Perspective of the Diagnostics Manufacturer – Next-Generation Sequencing for Personalized Medicine

Lynne McBride
Director, Regulatory Affairs & Clinical
Life Sciences Solution
Thermo Fisher Scientific

Regulatory Considerations for Multiplex Companion Diagnostic Test Development from the Pharmaceutical Perspective

Michael Benecky, PhD
Senior Director, Global Regulatory Affairs-Diagnostics
GlaxoSmithKline

Emerging Regulatory Paradigm for Next-Generation Sequencing

Xueying Sharon Liang, MD, PhD
Scientific Reviewer
CDRH/OIR/DMGP, FDA

12:30-1:30PM LUNCHEON AND NETWORKING

1:30-3:00PM SESSION 7: BIG DATA

SESSION CHAIR

Jeffrey Stuart, PhD, RAC
Director
Regulatory Affairs
Novartis Pharmaceuticals Corporation

Despite the rapid evolution in diagnostic multiplexing from systems with improving quality and standardization, only a fraction of the information generated is in use today for clinical decision-making. Scientists and regulators face significant challenges in deciding how to organize and utilize data that is being generated, and how to optimize its use to benefit patients. The ability to extract knowledge and insights from large and complex data sets in the drug development and clinical care settings is vital to driving the next breakthrough in the field of precision medicine. This session will explore common regulatory, technical and clinical hurdles to knowledge extraction, and highlight some novel approaches to overcome them.

Use of Phenotypic and Genotypic Data in Support of Precision Medicine and Learning Health Care Systems Activities: Approach of the Department of VA Affairs

Louis Fiore, MD, MPH
Executive Director
Massachusetts Veterans Epidemiology Research and Information Center
Professor of Medicine
Boston University

Applications for Cognitive Computing, Big Data and Advanced Analytics in Precision Medicine - A Case Study Approach

John Piccone, MD
Lead Partner Life Sciences Strategy and Analytics
Watson Solutions at IBM

High-Performance Integrated Virtual Environment (HIVE): FDA's Solution for Next-Generation Sequencing Data Analysis in Research and Regulatory Domains

Vahan Simonyan, PhD
Lead Scientist, HIVE Project Director
CBER, FDA

3:00-4:00PM SESSION 8: EMERGING US LEGISLATION AND POLICY IN PRECISION MEDICINE

SESSION CHAIR:

Gregory Daniel, PhD, MPH, RPh
Brookings Institute

Given promising approaches to the development and regulatory review of companion diagnostics, this session will explore the remaining policy and infrastructure challenges to further harnessing targeted therapies to improve patient care. What key stumbling blocks remain? How might current policy and legislative proposals – including the US House of Representatives' 21st Century Cures Initiative and the Obama Administration's Precision Medicine Initiative – help to address these challenges? Where could other policy efforts be best directed? Panelists will discuss current proposals and their impact on clinical development, regulatory science, patient and provider decision-making, and other policy areas where companion diagnostics may have a significant role.

PANELISTS:

Bray Patrick-Lake, MS
Director of Stakeholder Engagement
Clinical Trials Transformation Initiative

Jeff Allen, PhD
Executive Director
Friends of Cancer Research

Scott McGoohan, JD
Director, Science and Regulatory Affairs
Biotechnology Industry Organization

4:00PM CONFERENCE ADJOURNED