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BIOMARKERS & DIAGNOSTICS WORLD CONGRESS 2013

MAY 6 - 8, 2013 | LOEWS PHILADELPHIA HOTEL | PHILADELPHIA, PA

*The Leading Annual Meeting Dedicated to Biomarkers
and Diagnostics Research and Implementation*

DINNER COURSES:

Fit-for-Purpose Biomarker Assay
Development and Validation

Next-Generation Sequencing as
a Clinical Test

Laboratory-Developed Tests

CONFERENCE PROGRAMS:

MAY 6 - 7, 2013

**TRACK 1: TRANSLATIONAL
BIOMARKERS IN DRUG DEVELOPMENT**

**TRACK 2: CLINICAL ASSAY
DEVELOPMENT**

**TRACK 3: CANCER TISSUE
DIAGNOSTICS**

MAY 6 - 8, 2013

**TRACK 4: EXECUTIVE SUMMIT:
COMPANION DIAGNOSTICS**

MAY 7 - 8, 2013

**TRACK 5: BIOMARKERS FOR
PATIENT SELECTION**

TRACK 6: CANCER DRUG RESISTANCE

**TRACK 7: EXOSOMES AND
MICROVESICLES AS BIOMARKERS
AND DIAGNOSTICS**

FEATURED SPEAKERS



Nicholas C. Dracopoli
VP, Janssen R&D
Johnson & Johnson



Khusru Asadullah
VP, Head, Global Biomarkers
Bayer



Yoshi Oda
President, Biomarkers & Personalized Medicine
Eisai



Eric Lai
SVP, Head, Pharmacogenomics
Takeda



David Wholley
Director
Biomarkers Consortium



George Bashirians
Director, Clinical Research & Precision Medicine
Pfizer

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BIOMARKERS & DIAGNOSTICS WORLD CONGRESS 2013

CONFERENCE-AT-A-GLANCE

	Track 1: Translational Biomarkers in Drug Development	Track 2: Clinical Assay Development	Track 3: Cancer Tissue Diagnostics	Track 4: Executive Summit: Companion Diagnostics*
Sunday, May 5				
5:00-6:00	Conference Pre-Registration			
Monday, May 6				
8:30-10:00	Biomarkers in Translational Medicine	From Research Biomarkers to Clinical Assays	Whole-Slide Imaging and Digital Pathology	Commercialization of Companion Diagnostics
10:00-10:30	Networking Coffee Break			
10:30-11:50	Biomarkers in Translational Medicine	From Research Biomarkers to Clinical Assays	Whole-Slide Imaging and Digital Pathology	Commercialization of Companion Diagnostics
11:50-1:20	Luncheon Presentation <i>Sponsored by</i> Quanterix	Lunch on Your Own		
1:20-2:40	Biomarker Utility in Clinical Development	NGS in Clinical Use		Strategies for Rx-Dx Partnerships
2:40-3:40	Refreshment Break in the Exhibit Hall with Poster Viewing			
3:40-5:00	Biomarker Utility in Clinical Development	NGS in Clinical Use		Strategies for Rx-Dx Partnerships
5:00-6:00	Networking Reception in the Exhibit Hall with Poster Viewing			
6:00-9:00	Dinner Courses (Separate registration required) Fit-for-Purpose Biomarker Assay Development and Validation Next-Generation Sequencing as a Clinical Test			
Tuesday, May 7				
7:30-8:15	Breakfast Presentation <i>Sponsored by</i> MYRIAD  RBM			
8:25-10:00	Biomarkers for Safety Assessment	Choosing a Platform for Companion Diagnostics	Advances in IHC: Guiding Therapy Decisions	Choosing a Platform for Companion Diagnostics
10:00-11:00	Coffee Break in the Exhibit Hall with Poster Viewing			
11:00-12:15	Biomarker Collaborations and Consortia	Multiplexed Assays	Tissue Biomarkers for Targeted Therapy	Panel Discussion: Next-Generation CDx Platforms
12:15-1:45	Lunch on Your Own and Conference Registration for Tracks 5-7			
	Track 5: Biomarkers for Patient Selection	Track 6: Cancer Drug Resistance	Track 7: Exosomes and Microvesicles as Biomarkers and Diagnostics	
12:15-1:45	Conference Registration			
1:45-2:40	Biomarkers to Diagnostics		Exosome Biomarkers in Drug Development	Biomarkers to Diagnostics
2:40-3:45	Refreshment Break in the Exhibit Hall with Poster Viewing			
3:45-5:30	Molecular Profiling of Tumor Heterogeneity to Guide Therapy		Exosome Biomarkers in Drug Development	Timeline for CDx Development
6:00-9:00	Dinner Course (Separate registration required) Laboratory-Developed Tests			
Wednesday, May 8				
7:30-8:15	Breakfast Presentation (Sponsorship Opportunity Available) or Morning Coffee			
8:25-10:30	Advancing Personalized Medicine	8:05 Secondary Resistance to Targeted Cancer Therapy	Exosomes as Disease Markers	Advancing Personalized Medicine
10:30-11:30	Coffee Break in the Exhibit Hall with Poster Viewing			
11:30-12:45	Advancing Personalized Medicine	11:30-1:15 Resistance to Various Therapies: Cancer Does Not Discriminate	11:30-1:15 Exosomes as Novel Cancer Biomarkers	Advancing Personalized Medicine
12:45	Close of Conference	1:15 Close of Conference	1:15 Close of Conference	Close of Conference
				*Executive pricing registration required

BIOMARKERS & DIAGNOSTICS WORLD CONGRESS 2013

DISTINGUISHED FACULTY

Jason M. Aliotta, M.D., Assistant Professor, Medicine, Warren Alpert Medical School, Brown University

John L. Allinson, FIBMS, Vice President, Biomarker Laboratory Services, ICON Development Solutions

Maria E. Arcila, M.D., Department of Pathology, Memorial Sloan-Kettering Cancer Center

Khusru Asadullah, M.D., Vice President and Head, Global Biomarkers, Bayer Pharma AG

Jiri Aubrecht, Pharm.D., Ph.D., Senior Director, Safety Biomarker Group Lead, Drug Safety Research & Development, Pfizer

M.J. Finley Austin, Ph.D., Personalized Healthcare & Biomarker Strategy Director, AstraZeneca

Nazneen Aziz, Ph.D., Director, Molecular Medicine, Transformation Program Office, College of American Pathologists

Geoffrey Stuart Baird, M.D., Ph.D., Assistant Professor, Laboratory Medicine, University of Washington

George Bashirians, Ph.D., Director, Diagnostics Lead, Clinical Research and Precision Medicine, Worldwide R&D, Pfizer

Robert A. Beckman, M.D., External Faculty, Center for Evolution and Cancer, Helen Diller Family Cancer Center, UCSF; Executive Director, Clinical Development Oncology, Daiichi Sankyo Pharma Development

Darrell R. Borger, Ph.D., Co-Director, Translational Research Laboratory, Massachusetts General Hospital Cancer Center

Mark Broenstrup, Ph.D., Director, Biomarker and Diagnostics, R&D Diabetes Division, Sanofi

Michael Burczynski, Ph.D., Executive Director, Biomarker Technologies, Discovery Medicine and Clinical Pharmacology, Bristol-Myers Squibb

Claudio Carini, M.D., Global Clinical Immunology and Biomarkers Lead, Bioenhancement Development Unit, Pfizer

Luigi Catanzariti, Ph.D., Executive Director and Global Program Director, Diagnostics, Novartis

Carol Cheung, M.D., Ph.D., Department of Pathology, University Health Network

Atish Choudhury, M.D., Instructor in Medicine, Medical Oncology, Dana-Farber Cancer Institute

Seth Crosby, M.D., Director, Partnerships & Alliances, Washington University School of Medicine

Mark E. Curran, Ph.D., Vice President, Immunology Biomarkers, Janssen Research & Development

Stephen P. Day, Ph.D., Director, Medical Affairs, Hologic

Viswanath Devanarayan, Ph.D., Global Head, Exploratory Statistics, AbbVie, Inc

Luis Alberto Diaz, M.D., Associate Professor of Oncology, Johns Hopkins Sidney Kimmel Comprehensive Cancer Center

Max Diem, Ph.D., Professor, Chemistry and Chemical Biology, Northeastern University

Nicholas C. Dracopoli, Ph.D., Vice President, Janssen R&D, Johnson & Johnson

Crislyn D'Souza-Schorey, Ph.D., Professor, Biological Sciences, University of Notre Dame
Dominik Duelli, Ph.D., Assistant Professor, Cellular and Molecular Pharmacology, Rosalind Franklin University of Medicine & Science, Chicago Medical School

Daniel Edelman, Ph.D., Facility Head, Clinical Molecular Profiling Core, National Cancer Institute, NIH

Reyna Favis, Ph.D., Scientific Director, Clinical Research & Development, Janssen Pharmaceutical Companies of Johnson & Johnson

Andrea Ferreira-Gonzalez, Ph.D., Professor and Chair, Division of Molecular Diagnostics; Director, Molecular Diagnostics Laboratory, Department of Pathology, Virginia Commonwealth University

Andrew Fish, Executive Director, AdvaMedDx

Shirin Khambata Ford, Ph.D., Executive Director and Global Head, Oncology Correlative Sciences, Novartis

Herbert A. Fritzsche, Ph.D., Senior Vice President and CSO, Health Discovery Corporation

Felix Frueh, Entrepreneur-in-Residence, Third Rock Ventures

Steve Furlong, Ph.D., Safety Science Lead, AstraZeneca Clinical Development

George A. Green IV, Ph.D., Director, Pharmacodiagnosics, Bristol-Myers Squibb

Patrick Groody, Ph.D., Divisional Vice President, Research & Development, Abbott

Steven Gutman, M.D., MBA, Strategic Advisor, Myraqa

Abdel Halim, Pharm.D., Ph.D., DABCC-MDX, DABCC-TOX, DABCC-CC, FACB, Director, Clinical Biomarkers, Daiichi Sankyo Pharma Development

Sam Hanash, M.D., Ph.D., Director, McCombs Institute for Cancer Early Detection and Treatment, MD Anderson Cancer Center

Charles R. Handorf, M.D., Ph.D., Professor and Chair, Pathology and Laboratory Medicine, University of Tennessee

Amir Handzel, Ph.D., Associate Director, Translational and Clinical Sciences, OSI Pharma (Astellas)

Chunhai "Charlie" Hao, M.D., Ph.D., Associate Professor, Neuropathology Attending, Department of Pathology & Laboratory Medicine, Emory University School of Medicine

Madhuri Hegde, Ph.D., Associate Professor, Human Genetics; Senior Director, Emory Genetics Laboratory, Emory University

Philip Hewitt, Ph.D., Head, Early Non-Clinical Safety (Liver and Kidney), Merck Serono

Stephen M. Hewitt, M.D., Ph.D., Clinical Investigator, Laboratory of Pathology, National Cancer Institute, NIH

Holly Hilt, Ph.D., Head, Disease and Translational Genomics, Hoffmann-La Roche; Adjunct Professor, University of Medicine and Dentistry New Jersey

Fred H. Hochberg, M.D., Associate Professor, Neurology, Massachusetts General Hospital
Shidong Jia, Ph.D., Scientist, Oncology Biomarker Development, Genentech
Chris Jowett, General Manager, Commercial Operations, Abbott Molecular

Jingfang Ju, Ph.D., Co-Director of Translational Research, Pathology, Stony Brook University

Peter M. Kazon, General Counsel, American Clinical Laboratory Association

Eric Lai, Ph.D., Senior Vice President and Head, Pharmacogenomics, Takeda Pharmaceuticals International

Ira M. Lubin, Ph.D., Team Lead, Genetics Laboratory Research and Evaluation Branch, CDC

Johan Luthman, D.D.S., Ph.D., Senior Program Leader, Neuroscience & Ophthalmology Research & Development Franchise Integrator, Merck

Elaine Lyon, Ph.D., Medical Director, Molecular Genetics, ARUP Laboratories

Ron Mazumder, Ph.D., MBA, Global Head, Research and Product Development, Janssen Diagnostics, Janssen Pharmaceutical Companies of Johnson & Johnson

Duncan McHale, Ph.D., Vice President, Global Exploratory Development at UCB Pharma

Alan Mertz, President, American Clinical Laboratory Association

Yoshi Oda, Ph.D., President, Biomarkers and Personalized Medicine Core Function Unit, Eisai

Lorraine O'Driscoll, Ph.D., Associate Professor, Pharmacology; Director, Research, School of Pharmacy and Pharmaceutical Sciences, Trinity College Dublin

Carol S. Palackdharry, M.D., MS, Medical Director, ActiveHealth Management; Clinical Lead, Oncology Condition Analysis, Aetna

Saumya Pant, Ph.D., Research Fellow, Merck

Liron Pantanowitz, M.D., Associate Professor, Pathology and Biomedical Informatics, University of Pittsburgh Medical Center

Scott D. Patterson, Ph.D., Executive Director, Medical Sciences, Amgen

Sonia Pearson-White, Ph.D., Scientific Program Manager, Oncology, The Biomarkers Consortium, Foundation for the National Institutes of Health

Emanuel Petricoin III, Ph.D., Co-Director, The Center for Applied Proteomics and Molecular Medicine, George Mason University

Suso Platero, Ph.D., Director, Oncology Biomarkers, Janssen Pharmaceuticals

Mark Priebe, MT(ASCP)SBB, Managing Director, QualityStar Quality Consortium

Debra Rasmussen, MBA, Senior Director, Regulatory Affairs, Johnson & Johnson

Hallgeir Rui, M.D., Ph.D., Professor, Cancer Biology, Medical Oncology, and Pathology, Thomas Jefferson University

Kurt A. Schalper, M.D., Ph.D., Associate Research Scientist, Pathology, Yale School of Medicine

Stephen C. Schmechel, M.D., Ph.D., Associate Professor, Pathology, University of Washington School of Medicine

Robert Schupp, Ph.D., Executive Director, Diagnostics Hematology/Oncology, Celgene Corporation

Jason S. Simon, Ph.D., Director, Immuno-Oncology Biomarkers, Discovery Medicine and Clinical Pharmacology, Bristol-Myers Squibb

Sharon Sokolowski, Ph.D., Principal Scientist, Pfizer Global Research & Development

Gyongyi Szabo, M.D., Ph.D., Professor, Gastroenterology, University of Massachusetts Medical School

Douglas D. Taylor, Ph.D., Professor, Obstetrics and Gynecology, University of Louisville School of Medicine

Meghna Das Thakur, Ph.D., Presidential Postdoctoral Fellow, Novartis Institutes for BioMedical Research

Emina Torlakovic, M.D., Ph.D., Associate Professor, Laboratory Medicine and Pathobiology, University of Toronto

Jessie Villanueva, Ph.D., Assistant Professor, Molecular & Cellular Oncogenesis Program, The Wistar Institute

Glen J. Weiss, M.D., Co-Head, Lung Cancer Unit, The Translational Genomics Research Institute (TGen); Director, Thoracic Oncology, Virginia G. Piper Cancer Center Clinical Trials at Scottsdale Healthcare; CMO, CRAB-Clinical Trials Consortium

David Wholley, Director, Biomarkers Consortium, Foundation for the NIH

David T.W. Wong, D.M.D., D.M.Sc., Professor, Associate Dean of Research, UCLA School of Dentistry and Director of Dental Research Institute

Janghee Woo, M.D., Albert Einstein Medical Center

Wen Jin Wu, M.D., Ph.D., Principal Investigator, Division of Monoclonal Antibodies, Office of Biotechnology Products, Center for Drug Evaluation and Research, FDA

Brenda Yanak, Ph.D., Precision Medicine Leader, Clinical Innovation, Pfizer

Tammie Yeh, Ph.D., Molecular Biomarkers, Oncology Lead, Merck

Eunhee S. Yi, M.D., Consultant, Anatomic Pathology, Mayo Clinic; Professor, Pathology, Mayo Clinic College of Medicine

Malcolm York, MPhil, Director and Head, Clinical Pathology and Safety Assessment, GlaxoSmithKline R&D

Theresa Zhang, Ph.D., Associate Director, Exploratory and Translational Sciences, Merck

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MONDAY, MAY 6, 6:00-9:00 PM

Fit-for-Purpose Biomarker Assay Development and Validation

Instructors:

John L. Allinson, FIBMS, Vice President, Biomarker Laboratory Services, ICON Development Solutions

Viswanath Devanarayan, Ph.D., Global Head, Exploratory Statistics, AbbVie, Inc

This tutorial will provide recommendations on the "fit-for-purpose" best practices in the development and validation of biomarker assays for exploratory or advanced biomarker applications. Strategies for different applications at various phases of biomarker development will be described. Key elements in the method of development and validation will be illustrated with examples, including reference to standard material, sample stability and collection integrity, validation and QC samples, validity of reference standards, calibration curve fitting methods, method optimization and feasibility studies. Special challenges in protein biomarker assays will be discussed, including strategies for moving from biomarker panels in the exploratory phase to the few markers chosen to support clinical trials, cross-validation of biomarker assays, etc.

Outline:

1. Introduction: Nomenclature, types of biomarker methods/assays, method development and validation road-map, fundamental validity, similarity and differences from PK assays and diagnostic applications
2. Pre-analytical and bioanalytical elements: Target range, standards, validation and QC samples, stability, matrix effect, specificity and relative selectivity
3. Calibration curve model selection, evaluation and weighting
4. Method feasibility and optimization with precision profiles
5. Evaluation of some pre-study validation characteristics such as precision, bias, sensitivity and quantification limits
6. Use of sample controls for in-study performance monitoring and conformance testing among laboratories
7. Special considerations for multiplex assays, cross-validation of assays, etc.
8. Method comparisons

Next-Generation Sequencing as a Clinical Test: It Takes a Community

Instructors:

Nazneen Aziz, Ph.D., Director, Molecular Medicine, Transformation Program Office, College of American Pathologists

Madhuri Hegde, Ph.D., Associate Professor, Human Genetics; Senior Director, Emory Genetics Laboratory, Emory University

Next-Generation Sequencing (NGS) is used widely in clinical research for the discovery of disease-associated genes and the clinical community is beginning to embrace this technology for diagnostic testing. The rapid evolution of NGS technologies presents significant opportunities and challenges for researchers and clinicians for improving health outcomes, particularly with respect to an increased emphasis on personalized and preventive medicine. Adoption of NGS in the clinical laboratory setting requires the adoption of many processes and procedures, such as the analytic and clinical validation of the test, CLIA certification/CAP accreditation, standards for reference materials, availability for proficiency testing, and questions regarding reimbursement and informed consent. The success of NGS as a viable diagnostic modality depends on many branches of the health care community working together. This session will be informative and practical for the researcher and laboratorians who are considering launching NGS as a clinical test.

TUESDAY, MAY 7, 6:00-9:00 PM

Laboratory-Developed Tests

Regulatory Issues Facing Laboratory-Developed Tests

Peter M. Kazon, General Counsel, American Clinical Laboratory Association

The development of molecular diagnostics has been accompanied by a host of regulatory issues, including coding, billing and FDA issues. This session will review recent changes affecting these codes as well as the position of Medicare on how to pay for these tests and tests including an algorithm, also referred to as MAAs. It will review the latest developments from the FDA concerning whether such tests will require FDA pre-market approval or clearance, and what action FDA is likely to take in the future. It will also review other actions that affect these tests, such as the new MolDx program being overseen by Palmetto GBA, a Medicare contractor.

Laboratory-Developed Tests in the Genomic Medicine Era: Validation, Regulation and Challenges Faced by New Technologies and Clinical Applications

Andrea Ferreira-Gonzalez, Ph.D., Professor and Chair, Division of Molecular Diagnostics; Director, Molecular Diagnostics Laboratory, Department of Pathology, Virginia Commonwealth University

Laboratory-developed tests are those tests developed, validated and performed by clinical laboratories. There are standards and regulations in place for the validation of these tests before they are introduced into clinical practice. This presentation will discuss the process of validation under the current regulatory framework, and regulatory challenges posed by new technologies such as NGS and its clinical applications.

LDTs in the Context of CLIA: An NIH Experience

Daniel Edelman, Ph.D., Facility Head, Clinical Molecular Profiling Core, National Cancer Institute, NIH

The mission of the Clinical Molecular Profiling Core (CMPC) of the National Cancer Institute (NCI) is to provide state of the art genomic testing for specimens obtained from NCI clinical trials. The greatest impact is effected where test results have immediate clinical application for personalized cancer care for individual patients enrolled in these trials. To that end, the CMPC is CLIA certified and provides a growing set of clinical test modalities. In this talk we'll discuss the challenges of meeting CLIA regulations in this new age of genomics at NIH for high-complexity assays that did not exist as diagnostic tests when the federal guidelines were written.

LDT Regulation Guidance from the FDA: Where Does It Stand after Three Years?

Stephen P. Day, Ph.D., Director, Medical Affairs, Hologic

The FDA's announced intent to further regulate laboratory developed tests (LDTs) enters its third year without the issuance of the anticipated guidance. This presentation will summarize what the FDA has made publicly available on the subject up to this time, the positions and recommendations put forward by medical and industry professional societies, and how it will potentially affect clinical laboratories offering LDTs and the delivery of quality medical care.

Next-Generation Sequencing Assays as Laboratory-Developed Tests

Elaine Lyon, Ph.D., Medical Director, Molecular Genetics; Co-Medical Director, Pharmacogenomics, ARUP Laboratories; Associate Professor, University of Utah

As next-generation sequencing (NGS) technologies improve in accuracy and cost effectiveness, they will become standard in clinical laboratories. Multi-gene panels, exome or genome analysis are alternative approaches. With the complexity of genomic scale sequencing, implementing NGS assays into clinical laboratories requires expertise in laboratory techniques, informatics and interpretation. CLIA-certified clinical laboratories are developing NGS assays as laboratory-developed tests (LDTs). The presentation will discuss how NGS assays are "procedures" involving input from health care professionals, and how they fit under the category of high complexity LDTs.

*Separate registration required

TRACK 1: TRANSLATIONAL BIOMARKERS IN DRUG DEVELOPMENT

SUNDAY, MAY 5

5:00-6:00 pm Conference Pre-Registration

MONDAY, MAY 6

7:30-8:30 am Conference Registration and Morning Coffee

8:30-8:40 Welcome Remarks from Conference Director

Julia Boguslavsky, Executive Director, Conferences, Cambridge Healthtech Institute

Biomarkers in Translational Medicine

8:40-8:45 Chairperson's Opening Remarks

8:45-9:10 Translating Biological Data into Predictive Biomarker Development Strategies

Nicholas C. Dracopoli, Ph.D., Vice President, Janssen R&D, Johnson & Johnson

A decade after completion of the human genome sequence, the translation of complex genomic data into widely used clinical tests has been slower than anticipated. Three complex tests (*in vitro* diagnostic multiplex index assays - IVDMA) have been approved as prognostic tests, but there has still not been a single approval of an IVDMA to predict response to therapy. Retrospective analyses of the development of predictive biomarkers for first-in-class oncology drugs over the last ten years shows that 1) insufficient patients have been exposed to an efficacious dose to support complex statistical analyses to correlate high-content data against clinical endpoints, and 2) biomarkers that correlate to response in Phase II studies are not always good predictors of overall survival in Phase III trials. We will need to modify the clinical development paradigm for first-in-class agents to support the efficient co-development of predictive markers.

9:10-9:35 Application of Next-Generation Sequencing in Phase III Oncology Trials

Shirin Khambata Ford, Ph.D., Executive Director and Global Head, Oncology Correlative Sciences, Novartis

Analysis of tumor samples by next-generation sequencing (NGS) has increased dramatically in the last 2 years. Most of the reported results are genetic landscapes generated on samples collected outside clinical trials or from early phase trials. Application of this technology in large global Phase III trials provides an excellent opportunity for treatment efficacy predictive biomarker explorations. Study design considerations and analysis strategies for the implementation of complex and resource demanding NGS analysis in Phase III trials will be discussed.

9:35-10:00 Can Biomarkers Recover Drug Development from the Ditch?

Abdel Halim, Pharm.D., Ph.D., DABCC-MDx, DABCC-TOX, DABCC-CC, FACB, Director, Clinical Biomarkers, Daiichi Sankyo Pharma Development

Despite all the potential benefits of using biomarkers to advance the pharmaceutical industry, discrepant results can pose a threat to development programs by triggering false decisions. This talk will highlight the following topics: biomarkers and their potential utility in drug development, limitations, major reasons behind discrepant results and possibility of its mitigation.

10:00-10:30 Networking Coffee Break

10:30-10:55 Advancing Biomarkers for Alzheimer's Disease – From Target Engagement to Diagnostics

Johan Luthman, D.D.S., Ph.D., Senior Program Leader, Neuroscience & Ophthalmology Research & Development Franchise Integrator, Merck

Measuring pathophysiology associated factors, such as A β peptide and tau protein in cerebrospinal fluid, and imaging brain function with fluorodeoxyglucose PET or functional MRI, or pathology with amyloid PET or MRI, allows us to detect and follow the progression of very early, pre-dementia stages of AD. While the use of pathophysiology associated biomarkers allows pharmacodynamics monitoring of putative disease modifying therapeutics, further qualification efforts are paving the way for diagnostic and prognostic readouts.

10:55-11:20 Developing Biomarkers to Predict Response to Therapies in Oncology and Autoimmune Diseases through Molecular Disease Sub Typing

Renée Deehan Kenney, Ph.D., Vice President, Research, Selventa

Molecular drivers of disease are manifested across multitudes of interrelated biochemical pathways rather than genomic variations alone. We systematically interrogated thousands of these potential disease drivers with patient data to generate gene expression biomarkers to predict therapeutic response. Two case studies are presented: a blood biomarker to select rheumatoid arthritis patients likely to respond to anti-TNFs, and a tumor biopsy biomarker to select ER+ breast cancer patients prone to disease progression during tamoxifen treatment.

11:25-11:40 Highly Multiplexed SOMAmer Assays as a Flexible Platform for Biomarker Discovery Research

Nick Saccomano, Ph.D., CTO, SomaLogic

SomaLogic presents a transformative proteomic biomarker discovery technology that measures > 1100 human proteins in just 50 μ L of a biological sample with high-performance and high-throughput. Average LOD is ~40 fM, the overall dynamic range spans 8 logs, with ~5.1 % coefficient of variation. This technology is enabled by a new class of reagents (termed "SOMAmers") that contain novel chemically modified nucleotides which greatly expand their physicochemical diversity. Our assay has been used in dozens of clinical and preclinical studies; we have also demonstrated the progression of protein signatures to multi-analyte panel assays for later stage applications.

11:40-11:55 Sponsored Presentation

(Opportunity available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

11:55-1:25 pm Luncheon Presentation

Obtaining NAT Sensitivity with ELISA: Results from Application of Simoa to Blood Screening

David Wilson, Ph.D., Vice President, Product Development, Quanterix

Until recently, nucleic acid testing (NAT) represented the most sensitive method for early acute HIV infection, when individuals are most contagious. Using Single Molecule Arrays (Simoa), a digital ELISA technique, researchers were able to demonstrate a 3000x sensitivity improvement over conventional ELISA and equivalence with the NAT gold standard but at a fraction of the cost. This ground-breaking research has significant implications for blood banking, HIV detection and beyond.

Biomarker Utility in Clinical Development

1:25-1:30 Chairperson's Remarks

1:30-1:55 Implementing Biomarkers in Clinical Trials

Suso Platero, Ph.D., Director, Oncology Biomarkers, Janssen Pharmaceuticals

Finding biomarkers is relatively easy nowadays. One has only to open any journal and find dozens of articles showing the discovery of new biomarkers. The bottleneck in the development of biomarkers is in the correlation of the appropriate biomarkers to each specific drug. This is done in the context of clinical trials. Several strategies will be presented of how to better accomplish this task in an efficient and time sensitive manner.

1:55-2:20 Clinical Innovation in Precision Medicine

Brenda Yanak, Precision Medicine Leader, Clinical Innovation, Pfizer

This presentation will give examples of how Pfizer is innovating in the clinical development space to aid in the advancement of precision medicine.

2:20-2:45 Discovering Oncology Biomarkers and Translating into Clinical Trials

Theresa Zhang, Ph.D., Associate Director, Exploratory and Translational Sciences, Merck

This talk will present a platform for discovering oncology response biomarkers using a large panel of tumor cell lines, validating them in selected *in vivo* models, and refining and estimating biomarker prevalence in a large human tumor reference dataset. The predictive signature

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TRACK 1: TRANSLATIONAL BIOMARKERS IN DRUG DEVELOPMENT

will then be converted into an analytically validated assay that will be performed in a CLIA- or CAP-certified laboratory in order to enroll patients for clinical trials. The process will be illustrated by examples.

2:45-3:45 Refreshment Break in the Exhibit Hall with Poster Viewing

3:45-4:10 Biomarker Discovery for Immuno-Oncology Agents

Jason S. Simon, Ph.D., Director, Immuno-Oncology Biomarkers, Discovery Medicine and Clinical Pharmacology, Bristol-Myers Squibb

Tumor cells can use escape mechanisms to avoid or suppress the natural immune response, ultimately resulting in tumor growth; in fact, avoiding immune destruction is one of the emerging hallmarks of cancer. Therefore, understanding and dismantling key immune escape mechanisms ("checkpoints") is a key focus of immuno-oncology research. In concert with identifying agents to regulate the immune checkpoint is working to understand which tumor types and patient characteristics will respond best to this treatment approach. This talk will review our strategy to identify biomarkers which help support clinical development and commercialization strategies.

4:10-4:35 Accelerating and Personalizing Clinical Trials with Biomarkers and Adaptive Design, the I-SPY 2 Example

Sonia Pearson-White, Ph.D., Scientific Program Manager, Oncology, The Biomarkers Consortium, Foundation for the National Institutes of Health

I-SPY 2 is a unique clinical trial managed as a public/private partnership by the Foundation for the NIH (FNIH) Biomarkers Consortium. I-SPY 2 employs an innovative adaptive trial design testing multiple drugs in high-risk breast cancers in the neoadjuvant setting, and will advance the understanding of which drugs work best with tumor types with different biomarker profiles, and the drive toward personalized medicine.

4:35 Metabolomic Profiling for NMR Based Clinical Assay Development

Thomas O'Connell, Ph.D., Senior Director, Assay Research & Development, LipoScience, Inc.

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Metabolomic profiling yields a unique picture of the downstream phenotype taking into account genetic influences as well as environmental factors such as diet, lifestyle and the microbiome. In this presentation it will be shown how NMR technology is used in both the discovery and translation of biomarkers into the clinical laboratory. Applications include the prediction, diagnosis and prognosis of disease as well as the guidance of pharmaceutical interventions.

5:05-6:05 Networking Reception in the Exhibit Hall with Poster Viewing

6:05-9:05 Dinner Courses

Fit-for-Purpose Biomarker Assay Development and Validation Next-Generation Sequencing as a Clinical Test

(Separate registration required. See Page 4 for additional information.)

TUESDAY, MAY 7

7:30-8:15 am Breakfast Presentation

Identifying Non-Invasive Biomarkers of Smoking-Related Parenchymal Lung Disease (i.e. COPD and IPF) to Detect Subclinical Lung Disease

Ivan O. Rosas, M.D., Assistant Professor, Medicine Division, Pulmonary & Critical Care Medicine, Brigham & Women's Hospital, Harvard Medical School

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Biomarkers for Safety Assessment

8:25-8:30 Chairperson's Opening Remarks

8:30-8:55 Presentation to be Announced

8:55-9:20 Biomarkers of Acute Kidney Injury: From Pre-Clinical Species to Human Patients

Jiri Aubrecht, Pharm.D., Ph.D., Senior Director, Safety Biomarker Group Lead, Drug Safety Research & Development, Pfizer

Acute kidney injury provides a significant challenge to drug development. Recently, new biomarkers of acute kidney injury have been developed. In this presentation we will review the recent progress in applying emerging biomarkers of acute kidney injury across pre-clinical species and human subjects.

9:20-9:45 Preparing for Safety Biomarkers to Support Clinical Trials

Stephen T. Furlong, Ph.D., Safety Science Lead, AstraZeneca Patient Safety

Many new biomarkers are being considered for use in clinical trials to monitor drug-induced organ toxicity. However, deciding which biomarkers to use, selecting a vendor to perform the assays, establishing sample handling protocols, preparing for statistical analysis of the data and deciding how to use the data all represent significant challenges. This talk will review these topics, provide examples from specific biomarkers and provide suggestions for overcoming some of these challenges.

9:45-10:10 Identifying Biomarkers of Kidney and Liver Toxicity by Integrating Toxicogenomics Datasets with Biological Networks

Philip Hewitt, Ph.D., Head, Early Non-Clinical Safety (Liver and Kidney), Merck Serono

Candidate nephrotoxicity biomarkers were identified by interrogating profiles from hundreds of publicly available toxicogenomics datasets, including datasets from the EU PredTox and Japanese TG-GATES projects. Application of multiple bioinformatics approaches identified 43 significant candidates. These findings were corroborated by testing model nephrotoxic compounds using whole genome expression profile experiments both *in vivo* and *in vitro*. This *in silico* approach greatly enriched candidates for those likely to be true biomarkers.

10:10-11:00 Coffee Break in the Exhibit Hall with Poster Viewing

Biomarker Collaborations and Consortia

11:00-11:25 From Promise to Progress: An Update on the Biomarkers Consortium

David Wholley, Director, Biomarkers Consortium, Foundation for the NIH

11:25-11:50 Open Innovation in Biomarker Discovery: Experiences from Our Grants for Targets and Biomarkers Initiative

Khusru Asadullah, M.D., Vice President and Head, Global Biomarkers, Bayer HealthCare

To combine expertise Bayer Healthcare has set up a novel open innovation approach called Grants4Targets. After a review process, grants are provided to perform focused experiments to further validate the proposed targets/biomarkers. In addition to financial support, specific know-how on target validation and drug discovery is provided. Experienced scientists are nominated as project partners and, depending on the project, tools or specific models are provided. More than 600 applications have been received and 77 projects granted so far.

11:50-12:15 pm Biomarker Discovery—The Power of Collaborative Networks

Duncan McHale, Ph.D., Vice President, Global Exploratory Development, UCB Pharma

Clinically useful, predictive biomarkers have been very elusive despite the growth of Big Biology. Individual technology solutions are commonly touted as being able to identify drug response biomarkers but are rarely successful. It is likely that to be successful a network of collaborators will be needed bringing together technology discipline experts with disease biology experts. A case example is given in rheumatoid arthritis.

12:15 – 1:00 Luncheon Presentation

Speaker to be Announced

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TRACK 2: CLINICAL ASSAY DEVELOPMENT

SUNDAY, MAY 5

5:00-6:00 pm Conference Pre-Registration

MONDAY, MAY 6

7:30-8:30 am Conference Registration and Morning Coffee

8:30-8:40 Welcome Remarks from Conference Director

Julia Boguslavsky, Executive Director, Conferences, Cambridge Healthtech Institute

From Research Biomarkers to Clinical Assays

8:40-8:45 Chairperson's Opening Remarks

8:45-9:10 Biomarkers and the Quest for Clinical Utility—Obstacles, Challenges and Opportunities

Steven Gutman, M.D., MBA, Strategic Advisor, Myraqa

Over the past ten years there has been an explosive increase in the number of biomarker assays available for the study and evaluation of human disease. To ensure stakeholders are able to use this growing menu of tests responsibly, there is a compelling need to understand the clinical utility of these assays. Unfortunately a surprising number of tests are plagued by inadequate information on clinical utility. This talk will focus on obstacles, challenges and opportunities for addressing this problem.

9:10-9:35 Clinical Assay Development—The Process and Considerations

Herbert A. Fritsche, Ph.D., Senior Vice President and CSO, Health Discovery Corporation

The process for successful development of a clinical laboratory test begins with a strict definition of the test concept and its clinical utility; design of an accurate and robust assay for the analyte; analytical validation followed by clinical validation; and lastly, translation of the new test from the research lab to routine clinical use, which includes validation of the new test outside of the research setting. Success of development is defined as acceptance of the new test by the medical community as the "standard-of-care."

9:35-10:00 Bridging Research and "Clinical" Assays in Pharmaceutical Research & Development

John L. Allinson, FIBMS, Vice President, Biomarker Laboratory Services, ICON Development Solutions

Many biomarker assays used in drug development are research assays (i.e., not accredited diagnostic devices). This presentation will look at the following: basic validation experiments across assays in research and diagnostics; differences and assay evolution as methods progress through different uses of results data; the requirements for accreditation of assays to be used in diagnostics; and a brief look at the development of a companion diagnostic and its implications from the laboratory perspective.

10:00-10:30 Networking Coffee Break

10:30-10:55 Key Considerations for Choosing and Transitioning a Research Grade Assay to the Clinical Setting

Tammie C. Yeh, Ph.D., Molecular Biomarkers, Oncology Lead, Merck

Developing a biomarker assay with the clinical perspective in mind is critical to the success of the biomarker. Identifying/choosing a robust biomarker readout is as important as developing a robust analytical assay to ensure clinical utility. It is important to understand the inherent biological variability as well as the clinical feasibility of a biomarker readout, both of which will depend on tissue type, tissue processing and the specific clinical setting. Both patient selection and pharmacodynamic biomarkers will be addressed in this presentation.

10:55-11:20 Clinical Assay Development for Cancer Protein Biomarkers: What Works and What Does Not Work

Samir Hanash, Ph.D., Program Head, Molecular Diagnostics, Fred Hutchinson Cancer Research Center

The breadth and depth of proteomics technologies for the discovery of biomarkers has increased substantially over the past decade, covering a dynamic range of more than 7 logs in protein abundance. As a result, numerous cancer biomarker candidates have emerged from discovery studies. There remains a need for the development of high-throughput technologies that allow testing the utility of these biomarkers for their intended clinical application to meet regulatory requirements. Current opportunities and challenges will be presented.

11:20-11:45 Will Regulation of Laboratory-Developed Tests Stifle Innovation?

Alan Mertz, President, American Clinical Laboratory Association

Laboratory developed tests (LDTs) are regulated by Federal law (CLIA), state law, and industry standards established by the College of American Pathologists. For many years, FDA has maintained that LDTs are medical devices. FDA's legal authority has been questioned, however, and Congress, in July 2011, considered legislation that would enhance the CLIA framework for regulating LDTs. FDA's work plan for 2013 does not mention new guidance on LDTs, but remains a possibility. Closing the LDT pathway would have substantial effects on clinical laboratories, health care providers, and patients. This presentation will examine the role of LDTs in creating new tests, diagnosing rare diseases, and including the most up-to-date clinical information in diagnostic tests.

11:45-1:20 pm Enjoy Lunch on Your Own

NGS in Clinical Use

1:20-1:25 Chairperson's Remarks

1:25-1:50 College of American Pathologists' Standards and Proficiency Testing for Next-Generation Sequencing for the Clinical Laboratory

Nazneen Aziz, Ph.D., Director, Molecular Medicine, Transformation Program Office, College of American Pathologists

The rapid and ongoing advances in the genetic test market, spurred by the opportunities of Next-Generation Sequencing (NGS), necessitate many facets of the health care industry to work cohesively. Adoption of NGS as a clinical test requires the adoption of many processes and procedures, such as the analytic and clinical validation of the test, CLIA certification/CAP accreditation, standards for reference materials, availability for proficiency testing, genetic counseling, and questions regarding reimbursement, informed consent and incidental findings. This talk will focus on the laboratory requirements developed at CAP for CLIA/CAP accreditation and the plans for proficiency testing for NGS.

1:50-2:15 Assuring the Quality of Next-Generation Sequencing in Clinical Laboratory Practice

Ira M. Lubin, Ph.D., Team Lead, Genetics Laboratory Research and Evaluation Branch, Division of Laboratory Science and Standards, Laboratory Science, Policy, and Practice Program Office, Office of Surveillance, Epidemiology, and Laboratory Services, Centers for Disease Control and Prevention

Integration of next-generation sequencing (NGS) into the clinical laboratory requires test validation, establishment of quality control procedures, and the independent assessment of test performance by proficiency testing or alternate approaches. Existing regulatory requirements and professional guidance do not adequately address these quality issues for clinical NGS testing. This talk will describe the outcomes of a national workgroup organized by the Centers for Disease Control and Prevention tasked to identify principles and develop guidance to promote good clinical laboratory practices for NGS and meet regulatory and professional standards.

2:15-2:40 Clinical NGS: Validation, Reporting and Economics

Seth Crosby, M.D., Director, Partnerships & Alliances, Washington University School of Medicine

As NGS enters the clinic, matters of analytic and clinical validation are just the start of the medical director's worries. How should results be quickly generated and communicated to a physician in a meaningful and actionable manner? What are the new rules for billing and reimbursement?

2:40-3:40 Refreshment Break in the Exhibit Hall with Poster Viewing

3:40-4:05 Exome Sequencing in a Clinical Setting to Guide Patient Care

Madhuri Hegde, Ph.D., Associate Professor, Human Genetics; Senior Director, Emory Genetics Laboratory, Emory University

Advances in genomic medicine have made it necessary for clinical laboratories to rapidly implement new technologies to guide patient care. Exome sequencing is rapidly being implemented across different specialties such as inherited diseases, cancer and infectious diseases. This talk will focus on the clinical utility of exome sequencing in patient care with real case examples.

4:05-4:30 Interpreting Clinical Next-Generation Sequencing Data: Current Challenges and Hope for the Future

Elaine Lyon, Ph.D., Medical Director, Molecular Genetics; Co-Medical Director, Pharmacogenomics, ARUP Laboratories; Associate Professor, University of Utah

With the complexity of genomic scale sequencing (next-generation sequencing or NGS) and the massive amounts of data obtained, informatics is essential. Two challenges in evaluating a variant are 1) is it real and 2) is it clinically significant. Informatics allow alignment and variant calling (differences from a reference sequence), and sifting of probable clinically insignificant variants. More challenging is prioritizing variants that are likely to be associated with the clinical symptoms. In addition to the symptom-guided analysis approach, NGS data can reveal variants in genes related to drug metabolism that may affect efficacy or response. This presentation will discuss approaches to prioritize symptom-related variants as well as the potential of NGS data for companion diagnostics or therapeutics.

4:30-5:00 Assay and Kit Lot Bridging Considerations for Single and Multiplex Biomarker Analysis in Support of Clinical Studies

Afshin Safavi, Ph.D., Senior Vice President, Bioanalytical Operations, BioAgilytix Labs

Biomarker analysis has become a common practice by many pharmaceutical companies to help PK/PD modeling. The reliability of outcomes is heavily influenced by the quality of the reagents. One of the challenges that bioanalytical labs face when running biomarker studies is the control of lot-to-lot variability of critical reagents and commercial immunoassay kits. Case studies will be presented to highlight the key bioanalytical considerations involved in running successful biomarker analyses in support of clinical studies

5:00-6:00 Networking Reception in the Exhibit Hall with Poster Viewing

6:00-9:00 Dinner Courses

Fit-for-Purpose Biomarker Assay Development and Validation

Next-Generation Sequencing as a Clinical Test

(Separate registration required. See Page 4 for additional information.)

TUESDAY, MAY 7

7:30-8:15 am Breakfast Presentation

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Identifying Non-Invasive Biomarkers of Smoking-Related Parenchymal Lung Disease (i.e. COPD and IPF) to Detect Subclinical Lung Disease

Ivan O. Rosas, M.D., Assistant Professor, Medicine Division, Pulmonary & Critical Care Medicine, Brigham & Women's Hospital, Harvard Medical School

Choosing a Platform for Companion Diagnostics

8:25-8:30 Chairperson's Opening Remarks

8:30-8:55 Validating Biomarker Assays as a Prelude to Companion Diagnostic Development: Emerging Platform-Specific Considerations

Michael Burczynski, Ph.D., Executive Director, Biomarker Technologies, Discovery Medicine and Clinical Pharmacology, Bristol-Myers Squibb

Timely implementation of companion diagnostics alongside therapeutic products has amplified the need to validate predictive biomarkers in earlier phases of drug development. Today, biomarker strategies are more complex and require more diverse platforms than ever before. Ensuring that analytical validation strategies for these exploratory predictive biomarker assays are aligned with the downstream requirements for full-blown companion diagnostic development is a critical activity that ultimately helps determine the efficiency with which targeted medicines can be brought to market.

8:55-9:20 Choosing a Platform for Companion Diagnostic Development

Ron Mazumder, Ph.D., MBA, Global Head, Research and Product Development, Janssen Diagnostics, Janssen Pharmaceutical Companies of Johnson & Johnson

One of the early considerations in developing a companion diagnostic is choice of platform. Several factors, such as technical performance, regulatory and reimbursement path, and commercial access will be discussed in this context. Examples from the literature and case studies will be presented.

9:20-9:45 Thoughts and Considerations for Choosing a Companion Diagnostic Technology and Platform Delivery System

Patrick Groody, Ph.D., Divisional Vice President, Research & Development, Abbott

Choosing a diagnostic technology and testing platform for the development of a companion diagnostic test can be a significant challenge. A wide variety of factors including the development time, capabilities of potential partners and the ability of laboratories and physicians to access and perform the test routinely in a clinical setting are key factors in developing a companion diagnostic program. This talk will focus on variety of strategies for developing commercial companion diagnostic tests.

9:45-10:00 Sponsored Presentation

(Opportunity available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

10:00-11:00 Coffee Break in the Exhibit Hall with Poster Viewing

Multiplexed Assays

11:00-11:25 Measurement of Telomere Repeats in Human Cancer Cell Lines and Tissues Using a Monochrome Multiplex Quantitative PCR Assay

Daniel Edelman, Ph.D., Facility Head, Clinical Molecular Profiling Core, National Cancer Institute, NIH

This talk will describe our efforts for the development and validation of a QPCR multiplex assay to enable the quantitation of overall telomere length (TL) in cancerous cell lines and tissues. ATL pattern between cancers might provide valuable diagnostic or prognostic information to promote a better understanding of the molecular or pathogenic characteristics of specific cancer types.

11:25-11:50 Multiplexed Immunoassays on Formalin-Fixed, Paraffin-Embedded Tissue Homogenates as Cancer Diagnostics

Geoffrey Stuart Baird, M.D., Ph.D., Assistant Professor, Laboratory Medicine, University of Washington

Multiplex immunoassays (MIs) performed on formalin-fixed, paraffin-embedded (FFPE) tissue homogenates offer several advantages over immunohistochemistry as cancer diagnostics. In contrast to immunohistochemistry, MIs offer absolute quantitation and improved sensitivity and specificity through the use of sandwich assay geometries. Moreover, MI instrumentation has already been adopted in the clinical laboratory, and is much less expensive than a mass spectrometer. MIs have been validated as a clinical diagnostic for pituitary adenoma classification in FFPE tissue, with current work focused on breast carcinoma.

11:50-12:05 pm Diagnostic Classifiers for the Detection of Bladder Cancer

Mark Ruddock, Ph.D., Team Leader, Molecular Biology, Randox Pharma Services

Patients presenting with hematuria require investigations, including cystoscopy and imaging of their upper urinary tracts, to identify the source of bleeding. This is a significant health burden, which is set to increase because of our aging population. Using biochip array technology, we have identified diagnostic classifiers for detecting bladder cancer.

12:05-12:30 Development of Multiplexed Protein Pathway Activation Mapping Clinical Assays for Personalized Cancer Therapy

Emanuel Petricoin III, Ph.D., Co-Director, The Center for Applied Proteomics and Molecular Medicine, George Mason University

Cellular signaling pathways are a protein-based network, and the intended drug effect is to disrupt aberrant protein phosphorylation-based enzymatic activity, and epigenetic phenomena. The reverse-phase protein microarray platform provides detailed information about the state of the cellular "circuitry" from small samples. Measurements of dozens to hundreds of specific phosphorylated proteins that represent most of the targets for targeted therapeutics can be obtained at once from only a few thousand cells. This information helps select specific therapy(ies) tailored to the patient's tumor activated protein "circuitry."

12:30-1:45 Enjoy Lunch on Your Own

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RANDOX

TRACK 3: CANCER TISSUE DIAGNOSTICS

SUNDAY, MAY 5

5:00-6:00 pm Conference Pre-Registration

MONDAY, MAY 6

7:30-8:30 am Conference Registration and Morning Coffee

8:30-8:40 Welcome Remarks from Conference Director

Julia Boguslavsky, Executive Director, Conferences, Cambridge Healthtech Institute

Whole-Slide Imaging and Digital Pathology

8:40-8:45 Chairperson's Opening Remarks

8:45-9:10 Validation of Whole Slide Imaging in Pathology

Liron Pantanowitz, M.D., Associate Professor, Pathology and Biomedical Informatics, University of Pittsburgh Medical Center

Validation of whole slide imaging (WSI) is important to ensure that digitized slides are at least equivalent to that of glass slides. The College of American Pathologists (CAP) Pathology and Laboratory Quality Center convened a panel to recommend validation requirements for WSI systems to be used for clinical diagnostic purposes employing a combination of evidence-based evaluation of the literature, expert consensus and public commentary. The recommendations are comprehensive and address technical, interpretation components and administrative issues related to WSI in pathology providing practical guidance for all types of laboratories who are using or plan to utilize WSI systems for diagnostic clinical work. This session will educate participants about WSI in pathology, the regulatory issues surrounding digital pathology, and review the validation guidelines developed by the CAP.

9:10-9:35 New Applications Utilizing Whole Slide Digital Imaging for Anatomic Pathology Inter- and Intra-Lab Peer Review and Benchmarking Quality Assurance

Mark Priebe, MT(ASCP)SBB, Managing Director, QualityStar Quality Consortium

Although application of Whole Slide Digital Imaging (WSI) for primary diagnosis is limited by the FDA at this time, WSI is a significant enabling technology for anatomic pathology (AP) quality assurance (QA) initiatives both inter- and intra-laboratory. This presentation will review current AP/QA programs and the application of WSI to a novel approach of gaining longitudinal benchmarking data for quality review. The presentation will focus on understanding design requirements for development and implementation, investment requirements, confidentiality considerations and methods to encourage pathologist participation and acceptance.

9:35-10:00 Label-Free Infrared Spectral Histopathology: Diagnostics and Prognostics

Max Diem, Ph.D., Professor, Chemistry and Chemical Biology, Northeastern University

Infrared spectral histopathology is a method in which the biochemical composition of a histopathological sample is used, rather than morphometric criteria, to diagnose disease. To this end, thousands of infrared spectra are collected from pixels about 10 μm on edge, and analyzed to produce spectral images that detect abnormality based on variations in composition. The accuracy of this method is comparable to multi-panel immunohistochemistry.

10:00-10:30 Networking Coffee Break

10:30-10:55 Tumor Heterogeneity Assessed by Immunohistochemistry of Multiplexed Protein Biomarkers

Steve Schmechel, M.D., Ph.D., Associate Professor, Pathology, University of Washington School of Medicine

Intratumor heterogeneity of protein expression may be linked to the biological aggressiveness of tumors and selection of therapies. Analytical and statistical methods to quantify heterogeneity are needed, particularly for multiplexed assays. This presentation will discuss novel methods to measure tumor heterogeneity.

10:55-11:20 Application of WSI in Consensus Review for Clinical Trials

Stephen M. Hewitt, M.D., Ph.D., Clinical Investigator, Laboratory of Pathology, National Cancer Institute, NIH

Whole Slide Imaging is an enabling technology within pathology, altering all aspects of current practice. Consensus review processes for clinical trials have previously been expensive, slow, and complicated by issues of reproducibility. Whole Slide Imaging and distributed review overcome many of these issues, and provide new opportunities that have previously not been feasible.

11:20-11:50 Sponsored Presentations

(Opportunities available. Contact Ilana Quigley at 781-972-5457 or iqigley@healthtech.com)

11:50-1:20 pm Enjoy Lunch on Your Own

NGS in Clinical Use

1:20-1:25 Chairperson's Remarks

1:25-1:50 College of American Pathologists' Standards and Proficiency Testing for Next-Generation Sequencing for the Clinical Laboratory

Nazneen Aziz, Ph.D., Director, Molecular Medicine, Transformation Program Office, College of American Pathologists

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1:50-2:15 Assuring the Quality of Next-Generation Sequencing in Clinical Laboratory Practice

Ira M. Lubin, Ph.D., Team Lead, Genetics Laboratory Research and Evaluation Branch, Division of Laboratory Science and Standards, Laboratory Science, Policy, and Practice Program Office, Office of Surveillance, Epidemiology, and Laboratory Services, Centers for Disease Control and Prevention

Integration of next-generation sequencing (NGS) into the clinical laboratory requires test validation, establishment of quality control procedures, and the independent assessment of test performance by proficiency testing or alternate approaches. Existing regulatory requirements and professional guidance do not adequately address these quality issues for clinical NGS testing. This talk will describe the outcomes of a national workgroup organized by the Centers for Disease Control and Prevention tasked to identify principles and develop guidance to promote good clinical laboratory practices for NGS and meet regulatory and professional standards.

2:15-2:40 Clinical NGS: Validation, Reporting and Economics

Seth Crosby, M.D., Director, Partnerships & Alliances, Washington University School of Medicine

As NGS enters the clinic, matters of analytic and clinical validation are just the start of the medical director's worries. How should results be quickly generated and communicated to a physician in a meaningful and actionable manner? What are the new rules for billing and reimbursement?

2:40-3:40 Refreshment Break in the Exhibit Hall with Poster Viewing

3:40-4:05 Exome Sequencing in a Clinical Setting to Guide Patient Care

Madhuri Hegde, Ph.D., Associate Professor, Human Genetics; Senior Director, Emory Genetics Laboratory, Emory University

Advances in genomic medicine have made it necessary for clinical laboratories to rapidly implement new technologies to guide patient care. Exome sequencing is being rapidly implemented across different specialties such as inherited diseases, cancer and infectious diseases. This talk will focus on the clinical utility of exome sequencing in patient care with real case examples.

4:05-4:30 Interpreting Clinical Next-Generation Sequencing Data: Current Challenges and Hope for the Future

Elaine Lyon, Ph.D., Medical Director, Molecular Genetics; Co-Medical Director, Pharmacogenomics, ARUP Laboratories; Associate Professor, University of Utah

With the complexity of genomic scale sequencing (next-generation sequencing or NGS) and the massive amounts of data obtained, informatics is essential. Two challenges in evaluating a variant are 1) is it real and 2) is it clinically significant. Informatics allow alignment and variant calling (differences from a reference sequence), and sifting of probable clinically insignificant variants. More challenging is prioritizing variants that are likely to be associated with the clinical symptoms. In addition to the symptom-guided analysis approach, NGS data can reveal variants in genes related to drug metabolism that may affect efficacy or response. This presentation will discuss approaches to prioritize symptom-related variants as well as the potential of NGS data for companion diagnostics or therapeutics.

4:30-5:00 Sponsored Presentations

(Opportunities available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

5:00-6:00 Networking Reception in the Exhibit Hall with Poster Viewing

6:00-9:00 Dinner Courses

Fit-for-Purpose Biomarker Assay Development and Validation Next-Generation Sequencing as a Clinical Test

(Separate registration required. See Page 4 for additional information.)

TUESDAY, MAY 7

7:30-8:15 am Breakfast Presentation

Identifying Non-Invasive Biomarkers of Smoking-Related Parenchymal Lung Disease (i.e. COPD and IPF) to Detect Subclinical Lung Disease

Ivan O. Rosas, M.D., Assistant Professor, Medicine Division, Pulmonary & Critical Care Medicine, Brigham & Women's Hospital, Harvard Medical School

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Advances in Immunohistochemistry: Guiding Therapy Decisions

8:25-8:30 Chairperson's Opening Remarks

8:30-8:55 Quality Assurance/Quality Control for Immunohistochemistry in the Era of Personalized Medicine

Emina Torlakovic, M.D., Ph.D., Associate Professor, Laboratory Medicine and Pathobiology, University of Toronto

Immunohistochemistry (IHC) enables *in situ* detection of protein expression (right tissue, right cells, right cellular compartment) and evaluation of expression levels. Biomarker discovery increases demands on biomarker testing by IHC. IHC incorporates >20 parameters and requires expert interpretation. Key challenges include clinical trial study design, tissue processing parameters and parameters related to expert interpretation. IHC testing challenges remain significant due to widely spread lack of awareness that IHC QA/QC needs to evolve to match IHC intended use in personalized medicine.

8:55-9:20 Detection of ALK Gene Rearrangement (ALK+) in Non-Small Cell Lung Cancers

Eunhee S. Yi, M.D., Consultant, Anatomic Pathology, Mayo Clinic; Professor, Pathology, Mayo Clinic College of Medicine

Currently, ALK FISH is regarded as the gold standard to select the ALK+ patients eligible for crizotinib therapy, and FISH confirmation is required for "on-label" crizotinib treatment. ALK IHC can be useful to limit the number of patients to be tested for ALK FISH by identification of a high probability population whose tumors are likely to be ALK+. Current status of ALK IHC will be reviewed along with the data from a molecular study on discordant cases for ALK status by ALK IHC and FISH in a Mayo Clinic Lung Cancer Cohort.

9:20-9:45 Molecular Profiling and Immunohistochemistry: The Interface for Identification of Tissue of Origin in Occult Primary Cancers

Charles R. Handorf, M.D., Ph.D., Professor and Chair, Pathology and Laboratory Medicine, University of Tennessee

Metastatic tumors with an uncertain primary site can be a difficult clinical problem. In thousands of patients every year, no confident diagnosis is ever issued making standard-of-care treatment difficult. Newer gene expression profiling (GEP) tests currently available to analyze these difficult-to-diagnose tumors are now being compared head-to-head with immunochemistry (IHC), which has long been held as a gold standard. The interface between these techniques will be discussed and practical approaches will be explored.

9:45-10:00 Sponsored Presentation

(Opportunity available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

Tissue Biomarkers for Targeted Therapy

10:00-11:00 Coffee Break in the Exhibit Hall with Poster Viewing

11:00-11:25 *In situ* Measurement of Tissue Biomarkers for Companion Diagnostics in Cancer

Kurt A. Schalper, M.D., Ph.D., Associate Research Scientist, Pathology, Yale School of Medicine

Measurement of tissue biomarkers has been shown to be a valuable tool for companion diagnostics and is an essential component of personalized cancer medicine. Several technical limitations surround commonly used testing methods. *In situ* measurement of protein and mRNA transcripts using automated quantitative immunofluorescence and novel hybridization techniques provides increased sensitivity, specificity and reproducibility. More quantitative approaches could open new opportunities for biomarker discovery and patient selection for anti-cancer treatments.

11:25-11:50 Biomarkers and Targeted Therapy for Kaposi Sarcoma

Liron Pantanowitz, M.D., Associate Professor, Pathology and Biomedical Informatics, University of Pittsburgh Medical Center

Kaposi sarcoma (KS) is an enigmatic vascular neoplasm that arises from the initial infection of an endothelial or progenitor cell by Kaposi Sarcoma Herpesvirus/Human Herpesvirus-8 (KSHV/HHV8). KS represents an ideal model to investigate the interplay between viral oncogenesis, angiogenesis and host immunity. The discovery of KSHV and related data about the pathogenesis of KS has resulted in the identification of multiple novel therapeutic targets. This talk will educate participants about KS biomarkers being applied for diagnostic work, and also address newer therapeutic agents aimed at molecular targets being evaluated in clinical trials.

11:50-12:15 pm Access to Human Tissue in the Age of Targeted Therapies—Impact on Patient Care and Drug Development

Carol Cheung, M.D., Ph.D., Department of Pathology, Canadian University Health Network

Access to human tissue is paramount in this age of targeted therapies. Demand for this biological substrate, which is necessary for development of innovative new tests and potentially blockbuster new therapies, is ever increasing. The distinction between the two broad classes of excised human tissue, research tissue that resides in research biobanks and diagnostic tissue that resides in the clinical archives of institutional departments of pathology, is important because the rules governing access differ depending on this fundamental classification.

12:15-1:45 Enjoy Lunch on Your Own

TRACK 4: EXECUTIVE SUMMIT: COMPANION DIAGNOSTICS

SUNDAY, MAY 5

5:00-6:00 pm Conference Pre-Registration

MONDAY, MAY 6

7:30-8:30 am Conference Registration and Morning Coffee

8:30-8:40 Welcome Remarks from Conference Director

Julia Boguslavsky, Executive Director, Conferences, Cambridge Healthtech Institute

Commercialization of Companion Diagnostics

8:40-8:45 Chairperson's Opening Remarks

8:45-9:10 Companion Diagnostic Success: Biomarker Discovery to Global Commercialization

Chris Jowett, General Manager, Commercial Operations, Abbott Molecular

Developing a successful global commercialization strategy for a companion diagnostic can be a significant challenge. Critical capability factors need to be discussed prior to entering into the partnership to minimize risk. Understanding the IVD manufacturers' capabilities to develop, manage the required clinical trials, navigate the regulatory environment for approval, and drive sales and marketing efforts in all targeted countries for the therapeutic launch is essential. This talk will focus on a variety of strategies to support a successful launch of a companion diagnostic program.

9:10-9:35 The Payor's Role in Personalized Medicine

Carol S. Palackdharry, M.D., MS, Medical Director, ActiveHealth Management; Clinical Lead, Oncology Condition Analysis, Aetna

Targeted cancer treatment is already changing the standard of care for many cancers. Personalized therapies are costly and generally have anti-tumor activity only in patients with the specific targeted abnormality. Most targeted agents require pre-certification, with coverage dependent on appropriate results on approved companion diagnostic tests. Development of rigorous, evidence-based recommendations for usage of such tests, as well as new contracting strategies with high-quality laboratories, will avoid wasted expenditures and assure access to personalized therapies for all qualified patients.

9:35-10:00 Meeting Evidence Demands for Diagnostics in an Evolving Payment Environment

Andrew C. Fish, Executive Director, AdvaMedDx

Payer reimbursement of diagnostics is critical to ensuring a robust market for innovation. As advanced molecular diagnostics proliferate, a growing appreciation of the importance of these tests is tempered by rising payer concerns about coding transparency, evidence of clinical utility, and utilization of and payment for these tests. This talk will review the reimbursement challenges faced by test developers and initiatives underway by payers and in Congress to address those challenges.

10:00-10:30 Networking Coffee Break

10:30-10:55 Creating a Companion Diagnostic Regulatory Strategy: Biomarker to Commercial Test

Debra Rasmussen, Senior Director, Regulatory Affairs, Johnson & Johnson

Validated biomarkers (diagnostic tests) that can serve as intermediate or surrogate endpoints to acquire rapid regulatory approval are needed to help move research into the clinic. This is especially true if such biomarkers could be measured easily, rapidly and were generally accessible. Pharmaceutical companies could gain from biomarkers and diagnostic co-development efforts. In an increasingly challenging regulatory environment, diagnostic led treatment can improve the chance that drugs are reimbursed or approved in the first place. As companion diagnostics these could also potentially identify patient benefits from a novel therapeutic strategy earlier, assist in early discontinuation of ineffective strategies, and identify active drugs more efficiently. New concerns could include: 1) designing a definitive clinical study for a joint therapeutic-diagnostic that allows for assessment of the therapeutic's safety and efficacy, as well as for validation of the clinical utility of the biomarker guiding the therapeutic's use or 2) regulatory bodies requiring a diagnostic test before a prescription may be written for a patient.

10:55-11:25 Sponsored Presentations

(Opportunities available. Contact Ilana Quigley at 781-972-5457 or iqigley@healthtech.com)

11:25-11:50 Panel Discussion: Strategies for Regulatory and Reimbursement Challenges in Commercialization of CDx

Panelists:

*Chris Jowett, General Manager, Commercial Operations, Abbott Molecular
Carol S. Palackdharry, M.D., MS, Medical Director, ActiveHealth Management;
Clinical Lead, Oncology Condition Analysis, Aetna
Debra Rasmussen, Senior Director, Regulatory Affairs, Johnson & Johnson
Andrew C. Fish, Executive Director, AdvaMedDx*

11:50-1:20 pm Enjoy Lunch on Your Own

Strategies for Rx-Dx Partnerships

1:20-1:25 Chairperson's Remarks

1:25-1:50 Synchronizing Drug Development and Companion Diagnostics: Challenges and Solutions

George Bashirians, Ph.D., Director, Diagnostics Lead, Clinical Research and Precision Medicine, Worldwide R&D, Pfizer

Last year witnessed simultaneous regulatory approvals of Rx and Dx and it is expected that such approvals will be more commonplace in the near future. Synchronizing the drug development phases with those for Dx development presents many challenges. This talk will attempt to outline these challenges and offer solutions based on the Xalkori Rx/Dx program.

1:50-2:15 Managing Pharma/Diagnostic Partnerships in Companion Diagnostic Development

George A. Green IV, Ph.D., Director, Pharmacodiagnosics, Bristol-Myers Squibb

The development of CDx assays minimally requires a partnership between a pharmaceutical and a diagnostic company. It is not uncommon for the drug to be developed through an alliance of two pharmaceutical companies, and diagnostic assay development programs may include separate companies for design of the assay and development of the platform. To ensure effective delivery of the CDx within this complex environment, highly matrixed teams must be formed to address strategic and technical issues, and to deliver a quality product coordinated with the drug development schedule.

2:15-2:40 Presentation to be Announced

2:40-3:40 Refreshment Break in the Exhibit Hall with Poster Viewing

3:40-4:10 Key Considerations for Selecting a Diagnostic Partner in Rx-Dx Program Commercialization

*Sponsored by
Luminex*

Jeremy Bridge-Cook, Ph.D., Senior Vice President, Research & Development, Luminex

What are the optimal capabilities and expertise required of diagnostic partners for the development and commercialization of companion diagnostic devices? Key considerations include prototype assay development, analytical and clinical validation, regulatory filing, approval and market launch. The speaker will discuss how each of these elements can impact the success of a companion diagnostic program.

4:10-4:25 Sponsored Presentation

(Opportunity available. Contact Ilana Quigley at 781-972-5457 or iqigley@healthtech.com)

TRACK 4: EXECUTIVE SUMMIT: COMPANION DIAGNOSTICS

4:25-5:00 Panel Discussion: Strategies for Initiating and Managing Successful Rx-Dx Partnerships

Panelists:

George Bashirians, Ph.D., Director, Diagnostics Lead, Clinical Research and Precision Medicine, Worldwide R&D, Pfizer
George A. Green IV, Ph.D., Director, Pharmacodiagnosics, Bristol-Myers Squibb
Jeremy Bridge-Cook, Senior Vice President, Research & Development, Luminex

5:00-6:00 Networking Reception in the Exhibit Hall with Poster Viewing

6:00-9:00 Dinner Courses

Fit-for-Purpose Biomarker Assay Development and Validation Next-Generation Sequencing as a Clinical Test

(Separate registration required. See Page 4 for additional information.)

TUESDAY, MAY 7

7:30-8:15 am Breakfast Presentation

Sponsored by
MYRIAD RBM

Identifying Non-Invasive Biomarkers of Smoking-Related Parenchymal Lung Disease (i.e. COPD and IPF) to Detect Subclinical Lung Disease

Ivan O. Rosas, M.D., Assistant Professor, Medicine Division, Pulmonary & Critical Care Medicine, Brigham & Women's Hospital, Harvard Medical School

Choosing a Platform for Companion Diagnostics

8:25-8:30 Chairperson's Opening Remarks

8:30-8:55 Validating Biomarker Assays as a Prelude to Companion Diagnostic Development: Emerging Platform-Specific Considerations

Michael Burczynski, Ph.D., Executive Director, Biomarker Technologies, Discovery Medicine and Clinical Pharmacology, Bristol-Myers Squibb

Timely implementation of companion diagnostics alongside therapeutic products has amplified the need to validate predictive biomarkers in earlier phases of drug development. Today, biomarker strategies are more complex and require more diverse platforms than ever before. Ensuring that analytical validation strategies for these exploratory predictive biomarker assays are aligned with the downstream requirements for full-blown companion diagnostic development is a critical activity that ultimately helps determine the efficiency with which targeted medicines can be brought to market.

8:55-9:20 Choosing a Platform for Companion Diagnostic Development

Ron Mazumder, Ph.D., MBA, Global Head, Research and Product Development, Janssen Diagnostics, Janssen Pharmaceutical Companies of Johnson & Johnson

One of the early considerations in developing a companion diagnostic is choice of platform. Several factors, such as technical performance, regulatory and reimbursement path, and commercial access will be discussed in this context. Examples from the literature and case studies will be presented.

9:20-9:45 Thoughts and Considerations for Choosing a Companion Diagnostic Technology and Platform Delivery System

Patrick Groody, Ph.D., Divisional Vice President, Research & Development, Abbott

Choosing a diagnostic technology and testing platform for the development of a companion diagnostic test can be a significant challenge. A wide variety of factors including the development time, capabilities of potential partners and the ability of laboratories and physicians to access and perform the test routinely in a clinical setting are key factors in developing a companion diagnostic program. This talk will focus on variety of strategies for developing commercial companion diagnostic tests.

9:45-10:00 Sponsored Presentation

(Opportunity available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

10:00-11:00 Coffee Break in the Exhibit Hall with Poster Viewing

11:00-12:00 pm Panel Discussion: Next-Generation CDx Platforms

Panelists:

Michael Burczynski, Ph.D., Executive Director, Biomarker Technologies, Discovery Medicine and Clinical Pharmacology, Bristol-Myers Squibb
Ron Mazumder, Ph.D., MBA, Global Head, Research and Product Development, Janssen Diagnostics, Janssen Pharmaceutical Companies of Johnson & Johnson
Elaine Lyon, Ph.D., Medical Director, Molecular Genetics; Co-Medical Director, Pharmacogenomics, ARUP Laboratories; Associate Professor, University of Utah
Patrick Groody, Ph.D., Divisional Vice President, Quality Assurance and Operations, Abbott

12:00-1:45 Enjoy Lunch on Your Own

Biomarkers to Diagnostics

1:45-1:50 Chairperson's Remarks

1:50-2:15 Investing in Biomarkers and Turning Them into Diagnostics

Felix Frueh, Entrepreneur-in-Residence, Third Rock Ventures

The translation of biomarkers into useful clinical diagnostics requires the demonstration of clinical benefit and cost effectiveness. Investing in new technology is not sufficient without the realization that discovery and development of a new biomarker needs to include the demonstration that the biomarker makes a difference in clinical outcomes or decision-making, preferably tested in the environment in which ultimately a diagnostic will be used.

2:15-2:40 From Biomarker Research to Diagnostic Development—Our Challenges

Yoshi Oda, Ph.D., President, Biomarkers and Personalized Medicine Core Function Unit, Eisai

Biomarkers play important roles for drug development as a part of translational research. Several examples about biomarkers for 1) the evidence of target engagement, 2) patient stratification, 3) drug efficacy and 4) disease diagnostics will be discussed.

2:40-3:45 Refreshment Break in the Exhibit Hall with Poster Viewing

Timeline for CDx Development

3:45-3:50 Chairperson's Remarks

3:50-4:15 Timeline Considerations for Incorporating a Companion Diagnostic into the Drug Development Process

Luigi Catanzariti, Ph.D., Executive Director and Global Program Director, Diagnostics, Novartis

Rx/Dx co-development provides new opportunities for Pharma with respect to targeted therapeutics. It also comes with considerable clinical, technical and regulatory challenges. While both drug and diagnostic development processes have their own rules and regulations, this new codependency requires significant adjustments in what can be considered quintessentially clinical (Rx) and technical (Dx) development cultures. Mutual understanding and integration of both cultures early in the development process is an important aspect for minimizing development timelines and achieving success.

4:15-4:40 Nothing Ventured, Nothing Gained: The Timeline Challenge for Companion Diagnostics

Scott Patterson, Ph.D., Executive Director, Medical Sciences, Amgen

The identification of patients who are most likely to benefit from therapy is an important component of any drug development strategy. Other than when the target of the therapeutic is also the diagnostic for patient selection, the generation of evidence to test a biomarker patient selection hypothesis occurs during the drug development process. That data may not become available until late in the development process. Strategies that could be pursued to address this issue, with examples, will be presented.

4:40-5:05 Strategic and Computational Considerations in Development of Complex Companion Diagnostics

Amir Handzel, Ph.D., Associate Director, Translational and Clinical Sciences, OSI Pharma (Astellas)

Successful development of CDx requires special attention to diverse factors, as well as to their seamless integration. These challenges in developing validated complex diagnostic biomarkers have been highlighted by several failures in the last decade. The universe of molecular entities from which markers can be chosen is rich, comprising genetic mutations, the transcriptome, proteins and emerging non-coding RNA and epigenetic entities. Their extremely large numbers present difficult problems of selection and validation in a statistically robust and consistent way. In order to address them, an array of technical, as well as operational and organizational approaches must be employed. For example, the characteristics of the experimental platforms used to acquire data influence biomarker selection and design and these in turn necessitate a multidisciplinary team structure. I will discuss these strategic and technical elements while pointing to pitfalls and how to avoid them to reach the desired goal.

5:05-5:30 Companion Diagnostics: Challenges in Bridging the Chasm between Diagnostics and Drugs

Steven Gutman, M.D., MBA, Strategic Advisor, Myraqa

An IVD companion diagnostic device is an *in vitro* diagnostic device that provides information essential for the safe and effective use of a corresponding therapeutic product. This pairing of products has generated intense interest because 1) it offers a clear model for the implementation of personalized health care and 2) it may contribute to more informed choices about how to manage the pipeline for new drugs. This talk will focus on potential roadmaps for use in drug-diagnostic co-development.

6:00-9:00 Dinner Course

Laboratory-Developed Tests

(Separate registration required. See Page 4 for additional information.)

WEDNESDAY, MAY 8

7:30-8:15 am Breakfast Presentation or Morning Coffee

(Sponsorship opportunity available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

Advancing Personalized Medicine

8:25-8:30 Chairperson's Opening Remarks

8:30-8:55 Personalized Health Care: 'One Size Does Not Fit All' Applies to Patients and Products

M.J. Finley Austin, Ph.D., Personalized Healthcare & Biomarker Strategy Director, AstraZeneca

The essence of Personalized Health Care (PHC) is identifying, understanding and partitioning drug response variation to improve clinical outcomes. Existing PHC examples demonstrate diversity in source of variation, path to market as well as market delivery and uptake. Current examples will be used to elucidate the implications of differing sources and degrees of variation, clinical utility, and timing of discovery all have for clinical trial design, regulatory strategy and market delivery.

8:55-9:20 Molecular Subtyping of Patients for Drug Development

Eric Lai, Ph.D., Senior Vice President and Head, Pharmacogenomics, Takeda Pharmaceuticals International

While the concept of drug-diagnostic co-development (CDx) has been around for awhile, most companion diagnostics are still an afterthought and not an integrated component of drug development. To benefit from the full potential of CDx, we have to change the strategy of drug target identification from the single target approach to systematic understanding of a patient's disease phenotypes. I will discuss some of the potentials steps that we have made to the drug development process.

9:20-9:45 Co-Diagnostics in Autoimmune Disorders: Improving Outcomes in RA and IBD

Mark E. Curran, Ph.D., Vice President, Immunology Biomarkers, Janssen Research & Development

Rheumatoid arthritis and inflammatory bowel disease are severe immune diseases with significantly reduced quality of life for patients. Despite advances in treatment with the evolution of antibody and recombinant protein based therapeutics, there remains a significant unmet clinical need for new therapies and integrated treatment solutions. At Janssen we are focused on transforming therapy in these diseases by applying systems pharmacology, precision medicine principles and developing companion diagnostics to create new treatment paradigms. Our objective is to provide for higher response rates, deeper remission, early interception and eventually prevention of these diseases. Progress toward these objectives will be discussed.

9:45-10:15 Complex microRNA Signatures of Response and Resistance as Powerful Biomarkers

Sponsored by

E. Robert Wassman, M.D., CMO, Rosetta Genomics



10:15-10:30 Sponsored Presentation

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10:30-11:30 Coffee Break in the Exhibit Hall with Poster Viewing

11:30-11:55 Precision Medicine: Triumphs and Tribulations

Claudio Carini, M.D., Global Clinical Immunology and Biomarkers Lead, Bioenhancement Development Unit, Pfizer

The current model for drug development is failing. Failures often occur either during the phase II trials, where either the candidate drug did not meet the expected pharmacological requirements or the targeted drug mechanism did not play a role in the patients population studied. Thus, a new "personalized medicine" strategy is needed to develop predictive biomarkers to assist in the decision making process during the pre-clinical phase of drug development and use biomarkers as companion diagnostics for stratifying patients in hypothesis-driven clinical trials.

11:55-12:20 pm Towards Personalized Medicine in Metabolic Diseases

Mark Broenstrup, Ph.D., Director, Biomarker and Diagnostics, R&D Diabetes Division, Sanofi

Currently, more than 346 million people worldwide have diabetes. The identification of the most effective drug(s) for the individual patient is guided by a few selection criteria and a trial-and-error approach. Consequently, the introduction of personalized approaches, accounting for the heterogeneity of the disease, is regarded as a key enabler for improved health care. An overview on biomarkers for assessing risk, monitoring disease progression and predicting response to drugs is provided, with a focus on beta cell imaging and systems biology solutions. Finally, major public-private partnerships aiming at personalized solutions in diabetes will be highlighted.

12:20-12:45 Translating Molecular Targets for Cancer Therapeutics

Glen J. Weiss, M.D., Co-Head, Lung Cancer Unit, The Translational Genomics Research Institute (TGen); Director, Clinical Research, Cancer Treatment Centers of America; CMO, CRAB-Clinical Trials Consortium

The presentation will focus on why there is a push to individualize cancer therapy, past failures and successes, and how to define the tumor context of vulnerability (COV). The talk will also describe the steps from pre-clinical to new drug application and show how to optimize the drug development path with knowledge of biomarker-based COV.

12:45 Close of Conference

TRACK 5: BIOMARKERS FOR PATIENT SELECTION

TUESDAY, MAY 7

12:15-1:45 Conference Registration

Biomarkers to Diagnostics

1:45-1:50 Chairperson's Opening Remarks

1:50-2:15 Investing in Biomarkers and Turning Them into Diagnostics

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Molecular Profiling of Tumor Heterogeneity to Guide Therapy

3:45-3:50 Chairperson's Remarks

3:50-4:15 Liquid Biopsies to Monitor Response and Resistance to Targeted Therapies

Luis Alberto Diaz, M.D., Associate Professor of Oncology, Johns Hopkins Sidney Kimmel Comprehensive Cancer Center

The simplest hypothesis to account for the development of resistance to EGFR blockade is that rare cells with KRAS mutations pre-exist at low levels in tumors with ostensibly wild-type KRAS genes. Although this hypothesis would seem readily testable, there is no evidence in pre-clinical models to support it, nor is there data from patients. To test this hypothesis, we determined whether mutant KRAS DNA could be detected in the circulation of 28 patients receiving monotherapy with panitumumab, a therapeutic anti-EGFR antibody. The results suggest that the emergence of KRAS mutations is a mediator of acquired resistance to EGFR blockade and that these mutations can be detected in a non-invasive manner. They explain why solid tumors develop resistance to targeted therapies in a highly reproducible fashion.

4:15-4:40 Application of Clinical Tumor Genotyping in Targeted Cancer Therapy

Darrell R. Borger, Ph.D., Co-Director, Translational Research Laboratory, Massachusetts General Hospital Cancer Center

Multiplexed tumor genotyping has been offered as a physician-ordered clinical test at a major U.S. cancer center. Over 3,000 patients have been evaluated and these new capabilities have fostered a genotype-directed approach to clinical trial design. By testing a broad spectrum of tumor types, new molecular signatures have been revealed and mechanisms of *de novo* and acquired resistance to targeted therapies have been uncovered. This has provided the foundation for expanding clinical cancer genotyping approaches for personalizing cancer care.

4:40-5:05 Quantitative Tumor Protein Profiling for Therapy-Relevant Stratification of Breast Cancer Patients

Hallgeir Rui, M.D., Ph.D., Professor, Cancer Biology, Medical Oncology and Pathology; Scientific Director, Jefferson Breast Care Center; Program Leader, Biology of Breast Cancer, Kimmel Cancer Center; Co-Director, Pathology Translational Research Core, Thomas Jefferson University

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**6:00-9:00 Dinner Course
Laboratory-Developed Tests**

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TRACK 5: BIOMARKERS FOR PATIENT SELECTION

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12:45 Close of Conference

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Ilana Quigley – Business Development Manager
781-972-5457 | iquigley@healthtech.com

TUESDAY, MAY 8

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**6:00-9:00 Dinner Course
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WEDNESDAY, MAY 8

7:30-8:05 am Morning Coffee

Secondary Resistance to Targeted Cancer Therapy

8:05-8:30 Biomarkers and Trastuzumab Resistance

Wen Jin Wu, M.D., Ph.D., Principal Investigator, Division of Monoclonal Antibodies, Office of Biotechnology Products, Center for Drug Evaluation and Research, FDA

Trastuzumab is an anti-HER2 antibody indicated for the treatment of HER2-positive breast cancer. Approximately two-thirds of HER2-positive breast cancers show primary resistance to trastuzumab treatment, and a majority of patients who achieve an initial response to trastuzumab acquire resistance to trastuzumab within one year. However, there are no clinically useful predictive biomarkers that can be used in conjunction with HER2 expression to predict the outcome of trastuzumab treatment in the HER2-positive breast cancer patients. We recently found that the phosphorylation of HER2-Y1248 was associated with the sensitivity of trastuzumab treatment, suggesting that the phosphorylation status of HER2-Y1248 may be a predictive biomarker for trastuzumab treatment.

8:30-8:55 Resistance to MAPK Pathway Inhibitors in Melanoma: Insights and Future Challenges

Jessie Villanueva, Ph.D., Assistant Professor, Molecular and Cellular Oncogenesis Program, The Wistar Institute

The mitogen-activated protein kinase (MAPK) pathway is a key therapeutic target for melanoma due to its activation in the majority of tumors. Numerous small molecule inhibitors aimed at controlling MAPK activity, such as BRAF and MEK inhibitors, are currently undergoing clinical investigation. However, their therapeutic success is limited by the development of drug resistance. To develop effective therapies for melanoma patients, it is critical to uncover the mechanisms of resistance to BRAF and MEK inhibitors. This presentation will discuss recent studies on the molecular mechanisms of resistance to inhibitors of the MAPK pathway and potential strategies to treat drug-resistant melanomas.

TRACK 6: CANCER DRUG RESISTANCE

8:55-9:20 A Pre-Clinical Model of BRAF Inhibitor Resistance in Melanoma Reveals a Novel Approach to Forestall Drug Resistance

Meghna Das Thakur, Ph.D., Presidential Postdoctoral Fellow, Novartis Institutes for BioMedical Research

BRAF inhibitors such as vemurafenib have shown promising effects in patients with mutant BRAF(V600E) melanomas, but the tumors generally develop resistance. Interestingly, the vemurafenib-resistant melanomas become drug dependent for their continued proliferation, such that cessation of drug administration leads to regression of established drug-resistant tumors. Thus, a discontinuous dosing strategy exploiting the fitness disadvantage shown by drug-resistant cells in the absence of the drug forestalls the onset of lethal drug-resistant disease.

9:20-9:45 Non Cell-Autonomous Mechanisms of Resistance against Anti-EGFR Therapy

Janghee Woo, M.D., Albert Einstein Medical Center; Recipient of AACR-GlaxoSmithKline Clinical Cancer Research Scholar Award and Dana-Farber/Harvard Cancer Center Award

Our findings suggest that stroma-derived MMP9 may help tumors bypass common mutational mechanisms for constitutive growth factor pathway activation and confer resistance to anti-EGFR therapy through activation of the ERBB2/ERK/JUN pathway. Stromal MMP9 expression may therefore have value as a predictive marker for cetuximab response and in stratifying patients before treatment.

9:45-10:30 Sponsored Presentations

(Opportunities available. Contact Ilana Quigley at 781-972-5457 or iqigley@healthtech.com)

10:30-11:30 Coffee Break in the Exhibit Hall with Poster Viewing

11:30-11:55 Managing Secondary Drug Resistance in the Clinic: The Memorial Sloan-Kettering Approach

Maria E. Arcila, M.D., Department of Pathology, Memorial Sloan-Kettering Cancer Center

Resistance to Various Therapies: Cancer Does Not Discriminate

11:55-12:00 Chairperson's Remarks

12:00-12:25 A20 Ubiquitin E3 Ligase is a Biomarker of the Cancer Stem Cell Resistance to Apoptotic Drugs

Chunhai "Charlie" Hao, M.D., Associate Professor, Neuropathology Attending, Department of Pathology and Laboratory Medicine, Emory University School of Medicine

The TRAIL (tumor necrosis factor-related apoptosis-inducing ligand) apoptosis pathway has emerged as a cancer therapeutic target; however, Phase II trials recently completed have showed limited if any antitumor activities of TRAIL pathway-targeted therapies. Molecular and functional examination of patients' glioblastoma tissues and derived cancer stem cells reveals the resistance mechanism by which the ubiquitin E3 ligase A20 mediated poly-ubiquitination inhibits the cleavage of apoptosis-initiating caspase-8 and the initiation of TRAIL-induced apoptosis. The study suggests that the full characterization of patients' cancer tissues and derived cancer stem cells can predict the cancer responsiveness to treatment and thus should be a critical pre-clinical trial step in drug development.

12:25-12:50 pm Molecular Determinants of Hormone-Refractory Prostate Cancer

Atish Choudhury, M.D., Instructor in Medicine, Medical Oncology, Dana-Farber Cancer Institute

To identify novel genes that can confer androgen independence to prostate cancer cells *in vivo*, we performed an unbiased screen for kinases conferring androgen-independent tumor formation to androgen-dependent transformed prostate epithelial cells *in vivo*. These kinases are likely to activate signaling pathways that are relevant for conferring castrate resistance in patients with advanced prostate cancer, and inhibiting these genes is likely to result in inhibition of cancer cell proliferation and/or restoration of hormone sensitivity. Integration of our ambitious functional studies with gene expression and sequencing data in CRPC from tumor samples being generated through collaborations between DFCI and the Broad Institute will provide us a more comprehensive understanding of the development of castrate resistance and novel targets for therapy.

12:50-1:15 Impact of microRNAs in Chemoresistance

Jingfang Ju, Ph.D., Co-Director, Translational Research, Pathology, Stony Brook University

Non-coding miRNAs contribute to both intrinsic and extrinsic chemoresistance mechanism, particularly in colon cancer stem cells. We first discovered several miRNAs suppressing the expression of both thymidylate synthase and dihydrofolate reductase to impact 5-FU and MTX sensitivity. The expression of miR-215 was significantly associated with colorectal cancer patient survival. Our recent studies also show miRNAs impact intrinsic apoptotic pathways and autophagy. We believe miRNA based therapeutics, diagnosis and prognosis may emerge in the near future to benefit patients.

1:15 Close of Conference

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TUESDAY, MAY 8

12:15-1:45 Conference Registration

Exosome Biomarkers in Drug Development

1:45-1:50 Chairperson's Opening Remarks

1:50-2:15 Exosomes as Biomarkers for Translational Medicine

Holly Hilton, Ph.D., Head, Disease and Translational Genomics, Hoffmann-La Roche; Adjunct Professor, Graduate School of Biomedical Sciences, University of Medicine and Dentistry New Jersey

The need for new, relevant biomarkers for translational drug discovery research is critical. Exosomes are small microvesicles secreted by a wide range of mammalian cell types under normal and pathological conditions. The unique signature of exosomal membrane and cytoplasmic proteins as well as mRNAs and miRNAs can reveal the cell of origin and the condition of those cells. Isolation and profiling of exosomes from accessible patient biofluids, such as urine, blood, BALF and CSF, make them ideal candidates as biomarkers. Examples of their utility as disease biomarkers of chronic kidney disease and Alzheimer's as well as possible applications of patient stratification will be discussed. The current state of challenges to the widespread use of fluid-based biomarkers will be explored.

2:15-2:40 Investigation of Microparticles as Potential Translatable Biomarkers of Vascular Injury

Sharon Sokolowski, Ph.D., Principal Scientist, Pfizer Global Research & Development

Endothelial cells (EC) are thin, flattened cells that line blood and lymph vessel walls. Endothelial microparticles (EMPs) are small vesicles (0.1-1 µm) that are released into circulating blood from activated, injured or apoptotic endothelial cells and are found at elevated levels in a number of diseases associated with vascular/endothelial dysfunction. The EMPs are being investigated as potential translatable biomarkers of drug-induced vascular injury.

2:40-3:45 Refreshment Break in the Exhibit Hall with Poster Viewing

3:45-4:10 Utilization of Next-Gen Genomics Technologies for Unraveling Exosomal Biomarker Potential

Saumya Pant, Ph.D., Research Fellow, Merck

4:10-4:35 CNS Exosomes and the Art of Eavesdropping

Reyna Favis, Ph.D., Scientific Director, Janssen Pharmaceutical Companies of Johnson & Johnson

Gaining insight into both genomic changes and differences in the central nervous system of living humans is currently pursued via investigation of post mortem brain tissue and lymphocytes from living donors. Analyses of both tissue types suffer from numerous caveats. There is an urgent need to develop non-invasive methods that can accurately report temporal changes, as well as inter-individual differences, in the CNS that may elucidate neurological and neuropsychiatric disease and drug response.

4:35-5:00 Technology Assessment for Evaluation of Exosomal microRNA as Novel Biomarkers

Shidong Jia, Ph.D., Scientist, Oncology Biomarker Development, Genentech

Dr. Jia's lab has developed working procedures to evaluate exosomal microRNA as novel biomarkers for cancer prognosis, prediction and patient stratification. In particular, their work has refreshed current practice and demonstrated a new approach for studying microRNA signature in patient blood samples.

5:00-5:25 The Exosome Factor in Cancer

Lorraine O'Driscoll, Ph.D., Associate Professor, Pharmacology; Director, Research, School of Pharmacy and Pharmaceutical Sciences, Trinity College Dublin

Our research at Trinity College Dublin supports exosomes cargo having relevance as diagnostic, prognostic and predictive biomarkers. Evidence indicates they are also causative in cancer spread and drug resistance. Here we will discuss examples of this research in relation to breast cancer and prostate cancer.

**6:00-9:00 Dinner Course
Laboratory-Developed Tests**

(Separate registration required. See Page 4 for additional information.)

WEDNESDAY, MAY 8

7:30-8:15 am Breakfast Presentation or Morning Coffee

(Sponsorship opportunity available. Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com).

Exosomes as Disease Markers

8:25-8:30 Chairperson's Opening Remarks

8:30-8:55 Salivary Exosomes and Biomarkers Development

David T.W. Wong, D.M.D., D.M.Sc., Professor, Associate Dean, Research, UCLA School of Dentistry and Director of Dental Research Institute

Extracellular RNA is an emerging concept in cellular communication and biomarker development. Salivary extracellular RNA, microRNA and snoRNA have recently been shown to be contained within exosomes and can be developed to be discriminatory biomarkers for oral as well as systemic diseases.

8:55-9:20 Circulating Exosomes in Liver Disease

Gyongyi Szabo, M.D., Ph.D., Professor, Gastroenterology, University of Massachusetts Medical School

microRNAs (miRNAs) are fine tuners of diverse biological responses and are expressed in various cell types of the liver. They can also serve as biomarkers of liver damage and inflammation. We studied miRNA-122 that is abundant in hepatocytes and miR-155, -146a and -125b that regulate inflammation in immune cells in mouse models of various types of liver diseases and found that serum/plasma miR-122 correlated with ALT increases in the liver damage. miR-155, a regulator of inflammation, was increased in serum/plasma liver injury associated with inflammation. Depending on the type of liver injury, circulating miRNAs showed association either with the exosome-rich or protein-rich compartments. Our results suggest that circulating miRNAs may serve as biomarkers to differentiate between hepatocyte injury and inflammation and the exosome versus protein association of miRNAs may provide further specificity to mechanisms of liver pathology.

9:20-9:45 Microvesicles: Linking the Bone Marrow and Endothelium in Pulmonary Vascular Disease

Jason M. Aliotta, M.D., Assistant Professor, Medicine, Warren Alpert Medical School, Brown University

Extracellular vesicles (EVs) represent potentially important mediators of cell-to-cell communication and, depending on their source, facilitate tissue repair or remodeling. We've demonstrated that EVs isolated from mice with monocrotaline-induced pulmonary hypertension (PH) induce features of PH in normal mice. This may be due to EV-induced apoptosis resistance of pulmonary vascular endothelial cells or EV-induced differentiation of marrow cells into progenitor cells which, in turn, induce vascular remodeling. Conversely, we have found that mesenchymal stem cell-derived EVs may reverse monocrotaline-induced PH.

9:45 Sponsored Presentation

Speaker to be Announced

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10:15 Sponsored Presentation (Opportunity available.
Contact Ilana Quigley at 781-972-5457 or iquigley@healthtech.com)

10:30-11:30 Coffee Break in the Exhibit Hall with Poster Viewing

Exosomes as Novel Cancer Biomarkers

11:30-11:35 Chairperson's Remarks

11:35-12:00 pm The Exosome Platform as a Real-Time Tumor Status Monitor

Douglas D. Taylor, Ph.D., Professor, Obstetrics and Gynecology, University of Louisville School of Medicine

12:00-12:25 Customized Heterogeneity of Breast Cancer Microvesicles

Dominik Duelli, Ph.D., Assistant Professor, Cellular and Molecular Pharmacology, Rosalind Franklin University of Medicine & Science, Chicago Medical School

Breast cancer cells, unlike normal cells, release a heterogeneous population of circulating microvesicles. Resolving this heterogeneity suggests that individual microvesicle subclasses have different subcellular origin, different contents, and different destinations. Each subclass contains mutually exclusive, functional marker microRNA species, and some proteins with different functions in docking and lysis resistance in blood plasma. Additionally, organ-site of metastasis influences the ratio of these proteins, suggesting that these differences could be used to detect the presence of malignant cells in the body.

12:25-12:50 Tumor-Derived Microvesicles: Biology and Clinical Potential

Crislyn D'Souza-Schorey, Ph.D., Professor, Biological Sciences, University of Notre Dame

Tumor-derived microvesicles (TMVs) are heterogeneous membrane-bound sacs that are shed from tumor cells into the extracellular environment. The formation of these shed vesicles likely involves the vertical trafficking of intracellular cargo to the cell surface. The complexity of bioactive cargo contained in TMVs suggests multi-pronged mechanisms by which shed TMVs can condition the extracellular milieu to facilitate disease progression. It also demonstrates the potential to translate this knowledge into innovative approaches for cancer diagnostics and therapy.

12:50-1:15 Exosome Biomarkers of Brain Tumors

Fred H. Hochberg, M.D., Associate Professor, Neurology, Massachusetts General Hospital

We explore technology for detection of plasma and CSF exosomal mutations specific to brain tumors. The analytics for mutations EGFRvIII and IDH1.132 offer the potential to provide a diagnostic biomarker for low grade and high grade gliomas. An eighteen member consortium, collaborating with the ABC2 Foundation and the company Exosome Diagnostics, will validate the sensitivity of these biomarker assays. The presentation will include discussion of pre-clinical detection, SOPs for specimen handling and the rationale for use of these biomarkers.

1:15 Close of Conference

CONFERENCE HOTEL:

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Phone: 215-627-1200

Discounted Room Rate: \$229 s/d

Discounted Room Rate Cut-off Date: April 8, 2013

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May 6-7, 2013	May 7-8, 2013
Track 1: Translational Biomarkers in Drug Development	Track 5: Biomarkers for Patient Selection
Track 2: Clinical Assay Development	Track 6: Cancer Drug Resistance
Track 3: Cancer Tissue Diagnostics	Track 7: Exosomes and Microvesicles as Biomarkers and Diagnostics
Track 4: Executive Summit: Companion Diagnostics (May 6-8, 2013)	

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