Molecular Medicine
Tri-Conference
2010

Conference: February 3-5 | Exhibits: February 3-4
Moscone North Convention Center | San Francisco, CA

PLENARY KEYNOTES

When Drug Research is Personal
John F. Crowley, Founder, Novazyme Pharmaceuticals, Inc.

Technology, Aging, and the Brain
Gary W. Small, M.D., Professor, David Geffen School of Medicine, University of California, Los Angeles

Chips, Clones and Living Beyond 100
Paul J.H. Schoemaker, Ph.D., M.B.A., Professor, Wharton School of Business
# CONFERENCE-AT-A-GLANCE

## Tuesday, February 2

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>8:00 AM</td>
<td>Morning Short Course Registration and Coffee</td>
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<tr>
<td>9:00 - 12:00 PM</td>
<td><strong>Morning Short Courses (Courses 1-6)</strong></td>
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<tr>
<td>10:15 - 10:30</td>
<td>Networking Coffee Break</td>
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<tr>
<td>1:00 - 2:00</td>
<td>Afternoon Short Course Registration</td>
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<td>2:00 - 5:00</td>
<td><strong>Afternoon Short Courses (Courses 7-12)</strong></td>
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<tr>
<td>3:15 - 3:30</td>
<td>Networking Refreshment Break</td>
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## Wednesday, February 3

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<th>Time</th>
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<tbody>
<tr>
<td>7:00 AM</td>
<td>Registration and Morning Coffee</td>
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<tr>
<td>8:00 - 9:40</td>
<td>Plenary Keynotes</td>
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<tr>
<td>9:40 - 11:00</td>
<td>Grand Opening Refreshment Break in the Exhibit Hall</td>
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<td>11:00 - 12:40 PM</td>
<td><strong>Concurrent Channels</strong></td>
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<tr>
<td>12:40 - 1:45</td>
<td>Sponsored Luncheon Presentations or Lunch on Your Own</td>
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<tr>
<td>1:45 - 2:15</td>
<td>Dessert in the Exhibit Hall</td>
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<tr>
<td>2:15 - 4:20</td>
<td><strong>Concurrent Channels</strong></td>
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<tr>
<td>4:20 - 5:20</td>
<td>Reception in the Exhibit Hall (Sponsorship Available)</td>
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<td>5:20 - 6:20</td>
<td>Break-out Discussions</td>
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## Thursday, February 4

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<td>8:25 - 10:30 AM</td>
<td><strong>Concurrent Channels</strong></td>
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<tr>
<td>10:30 - 11:30</td>
<td>Poster Competition, Refreshment Break &amp; Raffles in the Exhibit Hall</td>
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<td>11:30 - 12:30 PM</td>
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<tr>
<td>12:30 - 1:45</td>
<td>Sponsored Luncheon Presentations or Lunch on Your Own</td>
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<tr>
<td>1:45 - 2:15</td>
<td>Ice Cream Refreshment Break in the Exhibit Hall</td>
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<tr>
<td>2:15 - 3:05</td>
<td>Plenary Keynote Session</td>
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<td>3:05 - 3:45</td>
<td>Refreshment Break in the Exhibit Hall</td>
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<td>3:45 - 5:50</td>
<td><strong>Concurrent Channels</strong></td>
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## Friday, February 5

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<td>8:30 - 10:20 AM</td>
<td><strong>Concurrent Channels</strong></td>
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<tr>
<td>10:20 - 11:00</td>
<td>Coffee Break</td>
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<tr>
<td>11:00 - 12:00 PM</td>
<td><strong>Concurrent Channels</strong></td>
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<tr>
<td>12:00 - 1:00</td>
<td>Sponsored Luncheon Presentations or Lunch on Your Own</td>
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<tr>
<td>1:00 - 3:05</td>
<td><strong>Concurrent Channels</strong></td>
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<td>Close of Conference</td>
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## CHI’s Intro-Net:

**Networking at Its Best!**

The Intro-Net offers you the opportunity to set up meetings with selected attendees before, during and after this conference, allowing you to connect to the key people that you want to meet. This online system was designed with your privacy in mind and is only available to registered session attendees of this event.

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**Online:** Tri-Conference.com  
**Email:** reg@healthtech.com  
**Fax:** 781-972-5425
PRE-CONFERENCE SHORT COURSES*

MORNING COURSES: 9AM – 12PM

(SC1) APPLYING NEXT GENERATION SEQUENCING TECHNOLOGIES TO RESEARCH
Introduction to New Technologies and Application in Research • Technologies for newest platforms for next generation sequencing • Strategies and tools for managing data • Demonstration of how tools can be applied to research
Course Moderator:
Stanley Glass, Founding Partner Managing Director, BioTeam, Inc.
Course Instructors:
Francisco M. De La Vega, D.Sc., Distinguished Scientific Fellow, Computational Genomics Research, Genetics Systems R&D, Life Technologies
Giles Day, Senior Director, BBC Informatics, Pfizer Biotherapeutics & Bioinnovation Center
Ronald W. Davis, Ph.D., Professor, Biochemistry & Genetics, and Director, Stanford Genome Technology Center, Stanford University

(SC2) ONE CASE STUDY IN BREAST CANCER: THREE PERSPECTIVES
Illustrating Current Challenges in Personalized Medicine
• Overview of case study • Clinical perspective • Diagnostic and biomarker perspective • Payer perspective
Course Instructors:
Michael Liebman, Ph.D., Managing Director, Strategic Medicine, Inc.
Laura J. Esserman, Professor, Surgery, University of California, San Francisco Medical Center
Tracey Colpitts, Ph.D., Manager, Abbott Molecular

(SC3) MIGHTY MITOCHONDRIA: Their Relevance to Disease and Translational Medicine
• Mitochondria in disease and drug induced toxicity (James Dykens) • Assessing mitochondrial function preclinically (Yvonne Will) • Non-invasive mitochondrial assessment in the clinic (Robert Wiseman)
Course Leader:
Yvonne Will, Ph.D., Compound Safety Prediction- WWMC, Cell Based Assays and Mitochondrial Biology, Pfizer R&D
Course Instructors:
James Dykens, Ph.D., Drug Safety R&D, Pfizer Inc
Yvonne Will, Ph.D., Compound Safety Prediction- WWMC, Cell Based Assays and Mitochondrial Biology, Pfizer R&D
Robert Wiseman, Ph.D., Associate Professor, Department of Physiology, Michigan State University

(SC4) ADDRESSING SAFETY CONCERNS FOR BIOLOGICAL DRUGS
• Overview of challenges pertaining to safety for biologics • Safety assessments at pre-clinical and clinical stage • Use of new assays, animal models and biomarkers for early predictions • Regulatory guidelines and their interpretations
Course Instructors:
Hong Wang, Ph.D., DABT, Safety Assessment, Genentech Inc.
Kathleen Meyer, MPH, Ph.D., DABT, Preclinical Safety Evaluation, XOMA (US) LLC

(SC5) TARGETING CANCER STEM CELLS WITH BIOLOGICS
• Novel nanoparticle fusion proteins, tr1 and tr4, that achieve normal p21 delivery to p53/p21 mutated tumors (tr1) and inhibition of notch signaling (tr4) resulting in tumor eradication • Differentiation versus self-renewal: changing cancer stem cell fate by targeting stem cell pathways. • Cancer stem-like cells: isolation using biological criteria and use in drug discovery and development • Cd47: an adverse prognostic factor and therapeutic antibody target on human acute myeloid leukemia stem cells • Identification of stem cell markers in the normal prostate and prostate cancer
Course Instructors:
Agamemon Epenetos, Ph.D., FRCPath, Chairman, Trojantree Ltd.
Austin Gurney, Ph.D., Vice President, Molecular and Cellular Biology, OncotMed Pharmaceuticals, Inc.
Jennie P. Mather, Ph.D., Senior Vice President, Stem Cell Research, MacroGenics, Inc.
Ravi Majeti M.D., Ph.D., Assistant Professor, Division of Hematology, Stanford Cancer Center, Institute for Stem Cell Biology and Regenerative Medicine
Kevin G. Leang, Ph.D., Scientist, Tumor Biology and Angiogenesis, Genentech, Inc.

(SC6) BLOOD-BRAIN BARRIER
• The physiological basis for the “barrier” nature of the BBB • Experimental approaches (in vitro/in vivo) that are available for screening for brain penetration • Medicinal Chemistry perspective on in vitro/in silico approaches for optimizing CNS penetration • Multi-parameter optimization (MPO) for CNS penetration • In vivo examples where all these concepts are applied together, e.g., consideration of free fractions in various compartments in relation to in vitro pharmacology values • Projecting human receptor occupancies considering species differences in affinity, free fraction • Exposure targeting for biomarker studies
Course Instructors:
Christopher L. Shaffer, Ph.D., Associate Research Fellow, Pharmacokinetics, Dynamics & Metabolism, Pfizer, Inc.
Douglas Sprodick, Ph.D., Director, Pharmacokinetics, Dynamics and Metabolism, Pfizer, Inc.
Travis T. Wager, Ph.D. Associate Research Fellow, Neuroscience Discovery, Medicinal Chemistry, Pfizer, Inc.

PRE-CONFERENCE SHORT COURSES*

AFTERNOON COURSES: 2PM – 5PM

(SC7) BEST PRACTICES IN TRANSLATIONAL & PERSONALIZED MEDICINE
• Real world solutions currently in place in pharma, national labs, academia, and industry • Building collaborations and sharing biological data between Big Pharma • Bridging the gap between bench and bedside • Informatics solutions that link data from the clinic with cutting edge research
Course Instructors:
Jeffrey S. Barrett, Ph.D., FCP, Pediatrics Director, Pediatric Pharmacology Research Unit, The Children’s Hospital of Philadelphia
Lisa LaLuna, Senior Vice President, Corporate Development & Implementation, ePharmaSolutions
Jeremy Packer, Head, Bioinformatics, Abbott
Taye D. Schillyer, Associate Director, NM Sequencing Center, National Center for Genome Resources

(SC8) STRATEGIES FOR MOLECULAR DIAGNOSTIC COMPANIES
Achieving Success in Rapidly Changing Markets • Why and how diagnostics markets have changed • Strategies for success: conventional or new markets? • Major business model questions including partnering • How to obtain your next round of funding
Course Instructors:
Keith F. Batchelder, Chief Executive Officer, Genomic Healthcare Strategies
Peter S. Miller, Chief Operating Officer, Genomic Healthcare Strategies

(SC9) FRAGMENT-INSPIRED MEDICINAL CHEMISTRY
• Fragment-based approaches as platforms for medicinal chemistry • Fragment-based methods that inspire fresh approaches to lead generation • Optimization of fragment hits • Combining technology with fragment-based methods to advance medicinal chemistry • Facing the challenge of applying fragment-based approaches when structural information is not available • Promises and pitfalls of surface plasmon resonance (SPR) for fragment methods
Course Instructors:
Michelle Arkin, Ph.D., Associate Director, Biology, Small Molecule Discovery Center, Pharmaceutical Chemistry, University of California, San Francisco
Daniel A. Erlanson, Ph.D., Co-founder, Carmot Therapeutics, Inc.

(SC10) TRANSPORTER-MEDIATED DRUG-DRUG INTERACTION POTENTIAL
Strategies for in vitro Characterization • Clinical relevance of transporter DDI’s • In vitro, cell based models for evaluating transporter interactions of substrates and inhibitors • Case study: minimizing p-glycoprotein interactions as a barrier to CNS penetration
Course Instructors:
Phil Burton, Ph.D., Chief Executive Officer & Chief Scientific Officer, ADMETRx, Inc.
Xiongeng Liu, Ph.D., Senior Scientist, DMPK, Genentech, Inc.
Joseph A. Ware, Ph.D., Senior Scientist, Clinical Pharmacokinetics and Pharmacodynamics, Development Sciences, Genentech, Inc.

(SC11) BASIC IMMERSION: CUTTING EDGE SCIENCE & TECHNOLOGY FOR BIOTECH & PHARMA
• Gain a fundamental understanding of the science and technology driving the Biotech/ Pharma industry • Learn basic scientific terminology used by researchers in the life sciences • Designed for the non-scientist working with or in the biotech/pharma industry • Immersion course on the biotech basics; Recombinant DNA, Proteins, Stem Cells, Biologics, Drug Discovery and Drug Development
Course Instructors:
Karin Lucas, Ph.D., BioTech Primer Instructor and Scientific Advisor

(SC12) DESIGNING RIGOROUS OMICS STUDIES FOR BIOMARKER DISCOVERY AND DEVELOPMENT OF PROGNOSTIC AND PREDICTIVE MOLECULAR DIAGNOSTICS
• Why study design is decisive for success or failure • Critical review of examples • Dos and don’ts • A roadmap to the answers • Samples: How many are enough? • Apples and Oranges: Tackling confounding factors • Sympathetic diagnostics in clinical trials • The regulatory perspective
Course Instructors:
Terry Speed, Ph.D., Professor, Department of Statistics, University of California, Berkeley
Juerger von Frese, Ph.D., Managing Director, Data Analysis Solutions, DA-Sol GmbH
Donna Rosce, Ph.D., Senior Reviewer, FDA/ODIV/DHID

Online: Tri-Conference.com  Email: reg@healthtech.com  Fax: 781-972-5425

Tuesday, February 2

*Separate Registration Required
4

CAMBRIDGE HEALTHTECH INSTITUTE’S SEVENTH ANNUAL

Molecular Diagnostics:
Next Wave of Personalized Medicine

WEDNESDAY, FEBRUARY 3

7:00 AM Registration and Morning Coffee
8:00 Plenary Keynote Session (See Page 26 for Details)
9:40 Grand Opening Refreshment Break in the Exhibit Hall

KEYNOTE PRESENTATIONS

11:00 Chairperson’s Remarks
Harry Glorikian, Managing Partner, Scientia Advisors

11:05 Molecular Diagnostics as a Value Driver of Pharma/Pharma as a Value Driver for Molecular Diagnostics
Michael C. Little, Global Head, Diagnostics Development, Novartis Molecular Diagnostics
Molecular Diagnostics is a critical success factor for the future of pharmaceuticals and an essential aspect of the move toward personalized medicine. At the same time, to progress better healthcare and patient outcomes, it is imperative that the pharma industry’s understanding of both targeted drug discovery and drug commercialization is fully leveraged to enable innovative diagnostics to be put into clinical practice and influence physician decision-making. The keynote will focus on these elements and discuss 1-2 case studies of how we at Novartis are using our discovery and development approach to work toward bringing innovative companion and stand-alone diagnostic tests to market.

11:40 Building a Successful Diagnostics Business Model in the Era of Personalized Medicine
Richard Ding, CEO, bioTheranostics, a bioMerieux Company Personalized medicine has been generally accepted as an inevitable trend in healthcare. However, much debate is still ongoing related to a sustainable business model for diagnostics companies in this new space. This presentation will identify various challenges, risks and potential returns for diagnostic companies, explore partnership models and propose some basic framework to seize the growth opportunity of personalized medicine.

12:15 PM Personalized Medicine: It Takes a Village
Mark Stevenson, President & COO, Life Technologies Corp.
New technologies, such as next generation sequencing, can be rapidly adopted in the research labs and help breakthroughs in our understanding of disease mechanisms for personalized medicine. But the journey from research technology to diagnostic systems is challenging and slow. As our understanding of disease increases the promise of personalized medicine is coming closer but what will it take to cross the bridge from research tool to routine diagnostics in personalized medicine. The presentation will focus on the journey Life Technologies has embarked on and the partnerships and collaborations necessary to translate the tools for the research lab into solutions personalized medicine.

12:50 Luncheon Presentation
Information Trends in Biomarker Research
Colin Williams, Ph.D., Director, Product Strategy
Thomson Reuters Healthcare and Science
In recent years, the quantity of data published on biomarker research has exploded. The challenge faced by researchers is to find vital, relevant information on the best biomarker quickly and reliably. In this discussion we will introduce BIOMARKERcenter, a comprehensive, fully-indexed biomarker information resource, and through case studies show how it aids the discovery process.

1:45 Dessert in the Exhibit Hall

MOLECULAR DETECTION OF PATHOGENS: MEETING THE NEEDS OF THE COMMUNITY

2:15 Chairperson’s Remarks
Karen L. Kaul, M.D., Ph.D., Board of Directors Chair of Molecular Pathology, Director, Molecular Diagnostics Division, Director, Pathology Residency Program, NorthShore University HealthSystem; Clinical Professor of Pathology, University of Chicago Pritzker School of Medicine

2:20 What Happened with SARS: Lessons Learned and Applied to Influenza
Lance R. Peterson, M.D., FASCP, FIDSA, Director, Microbiology & Infectious Disease, Evanston Hospital, NorthShore University HealthSystem and University of Chicago
Control of any epidemic relies on detection of those harboring the pathogen (infected and colonized). For any MRSA prevalence, the operational processes most influential are 1) sensitivity of the laboratory methods used, 2) speed at which unknown positive patients are detected, and 3) the selection of who is to undergo screening. The current understanding of these specifics will be presented.

3:20 MRSA Surveillance Programs – What Impacts Success?
Joseph D. Miller, Ph.D., Chief, Laboratory Preparedness Officer, Influenza Division, Centers for Disease Control and Prevention

3:50 MRSA Surveillance Programs – What Impacts Success?
Karen L. Kaul, M.D., Ph.D., Board of Directors Chair of Molecular Pathology, Director, Molecular Diagnostics Division, Director, Pathology Residency Program, NorthShore University HealthSystem; Clinical Professor of Pathology, University of Chicago Pritzker School of Medicine
The Novel H1N1 Influenza outbreak of 2009 challenged laboratories and hospitals with the need for high volume testing and rapid resulting in order to appropriately treat and isolate infected patients. Molecular approaches offer clear advantages, though no pre-validated assays were available for this unanticipated virus. This presentation will address various assays for detection and differentiation, as well as other laboratory issues, and will review the recent outbreak from the laboratory perspective.

2:50 What Happened with SARS: Lessons Learned and Applied to Influenza
Joseph D. Miller, Ph.D., Chief, Laboratory Preparedness Officer, Influenza Division, Centers for Disease Control and Prevention

3:50 Real-time Array PCR for Infectious Diseases(RAP-ID): Merging Multiplex PCR and Real-time Microarray Detection in a Single Tube for Sensitive Parallel Genotyping of Pathogens and Antibiotic Resistances
Wilhelm Pliester, Ph.D., CEO, Eppendorf Array Technologies S.A.
RAP is a novel hybrid technology combining major advantages of microarrays (multiplexing, specificity) and real-time PCR (sensitivity, dynamic range). Target amplification and hybridization of amplicons proceed in a single tube (in the same buffer) resulting in a simplified, automated workflow with minimal hands-on time. First results in multiplex detection of pathogens and antibiotic resistances associated with ventilator-associated pneumonia are presented.

4:05 Platform Genometrica:
Novel Instrumentation for Molecular Biology and Medicine
Vera Gorfinkel, Ph.D., Associate Professor, SUNY SB, Research consultant, Genometrica Corporation
The technology platform Genometrica aims to develop methods and instruments capable of carrying out on equal footing inexpensive and highly accurate genomic studies including DNA sequencing, hybridization, and quantitative PCR assays. The session will focus on the basic principles, novel engineering solutions, data acquisition/handling methods, and unique research capabilities offered by the Genometrica platform.

Sponsored by eppendorf Array Technologies
Sponsored by Genometrica

Industry leader’s networking event

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4:20 Reception in the Exhibit Hall (Sponsorship Available)

5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

Consumer Diagnostics (Not Just Genomics) – Get Used to It!
Co-Moderators: Peter S. Miller, Chief Operating Officer, and Keith F. Batchelder, Chief Executive Officer, Genomic Healthcare Strategies
- Technology improvements and cost reductions will make genetic, metabolic, and proteomic analysis cheaper
- Consumers will remain interested and companies will develop better ways of reaching the public
- Providers of traditional care will have to come to grips with informed consumers
- How will this happen?

Is Economics Going to Be the Driver of Molecular Diagnostics Adoption?
Moderator: David S. Lester, Ph.D., Vice President, Human Health Solutions, Theranos
- Are molecular diagnostics going to make healthcare cheaper and/or better?
- What are the barriers for adoption of molecular diagnostics?
- Fayers: US vs. international challenges.
- How will the value of the diagnostic be determined?

Patents and Diagnostics Development: Help or Hindrance?
Moderator: Frances Toneguzzo, Ph.D., Director, Office of Corporate Sponsored Research and Licensing, Massachusetts General Hospital
- Type of patents in the diagnostic space and IP fragmentation
- Other types of intellectual property protection in diagnostics development
- Differentiation in diagnostics and use of patents
- Strategies for effective use of intellectual property to stimulate diagnostic development

Making Molecular Diagnostics Work Now? The Trials and Tribulations of Labs and Money
Moderator: Ian S. Millett, Ph.D., RAC, Senior Consultant, Medical Devices, Biologics Consulting Group, Inc.
- How do you get paid for a molecular diagnostic test?
- Does implementing a molecular diagnostic test in your lab really make sense?
- Life at Ground Zero - The FDA’s changing perspective on lab-developed tests
- Pushing and Pulling: Who is your customer and just how personal can you make that bill?

Strategies for Commercialization of Molecular Diagnostics
Moderator: Harry Glorikian, Managing Partner, Scientia Advisors

6:20 Close of Day

THURSDAY, FEBRUARY 4

HEALTH IT: WHY IS IT SO HARD?

8:25 AM Chairperson’s Remarks
Wayne A. Rosenkranz, Jr., Ph.D., Distinguished Fellow, MIT Center for Biomedical Innovation; Program in Ethics and Systems Medicine, Georgetown University; Chairman, Personalized Medicine Coalition; VP, Strategic Consulting, Fulld & Co.; Chief Scientific Advisor, Expertech Solutions; and Chief Applications Officer, SciTech Strategies

8:30 Keynote Presentation
Ensuring Responsible Testing through Real-Time Collaboration Between Providers, Payors and Labs
Matthew B. Zubiller, VP and General Manager, Advanced Diagnostics Management, McKesson Corp.
As molecular diagnostics proliferate, ensuring responsible testing becomes more complex. This is further complicated by changing reimbursement policies and health care IT reform. Ensuring your lab’s success requires technology-enabled collaboration with providers, payors and other labs. This keynote discusses business practices and strategies for labs to offer decision support and access to a broader array of tests to providers, to review payors’ reimbursement policies before tests are performed and to build effective lab networks to fulfill orders.

9:15 Keynote Presentation
HIT and PM: Conflict or Convergence
Wayne A. Rosenkranz, Jr., Ph.D., Distinguished Fellow, MIT Center for Biomedical Innovation; Program in Ethics and Systems Medicine, Georgetown University; Chairman, Personalized Medicine Coalition; VP, Strategic Consulting, Fulld & Co.; Chief Scientific Advisor, Expertech Solutions; and Chief Applications Officer, SciTech Strategies

10:00 The Development of Multigene Prognostic and Predictive Tests in Cancer
Austin Tanney, Ph.D., Scientific Liaison Manager, Almac Diagnostics
The key to the delivery of personalized medicine is the development of molecular diagnostics to improve patient care, from better diagnostic and prognostic tests to companion diagnostics. The use of multigene signatures is increasingly of interest however there are many considerations in developing such signatures from study design to development of deliverable tests. Here we present out experience and perspective.

10:15 Sponsored Presentation (Sponsorship Opportunity Available)

10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

11:30 Expert Panel
Health IT: What Will Success Look Like?
Moderator: David S. Lester, Ph.D., VP, Human Health Solutions, Theranos
- Reducing healthcare costs
- Facilitating the adoption of molecular diagnostics
- Defining the goals
- Realizing solutions for reaching the goals
Panelists:
Brandon Savage, M.D., Chief Medical Officer, GE Healthcare
Vance Vanier, M.D., Chief Medical Officer, Navigenics, Inc.
Mark N. Blatt, M.D., MBA, Director, Healthcare Industry Solutions, Digital Health Group, Intel Corporation
Jeffrey D. Miller

12:30 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Ice Cream Refreshment Break in the Exhibit Hall
PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction

2:25 Plenary Keynote Presentation (See Page 26 for Details)

3:05 Refreshment Break in the Exhibit Hall

4:20 Gene Patents in Molecular Diagnostics: Valuable Assets or Impediments?
Frances Toneguzzo, Ph.D., Director, Office of Corporate Sponsored Research and Licensing, Massachusetts General Hospital
Increasingly, genetic diagnostics are making use of panels of genes/gene fragments for accurate diagnosis of drug responses (toxicity and/or effectiveness and/or dosing) and disease stratification. In a number of situations, the patents covering these genes or their use are held by different owners, including companies, academic institutions and private individuals/foundations. While patents are generally considered valuable in that they provide a period of exclusivity when a company can exclude others from practicing the patented invention and thus protect the investment the company is making in commercializing the invention, this fragmentation of the intellectual property landscape in molecular diagnostics may impede the development of certain tests.
Way to the Fair?

all costs of care.

ized Medicine healthcare can improve outcomes and reduce the over

become more conversant with the value of testing. Medco's Personal

standard of care in many therapeutic areas, as physicians and payers

characteristics of individual patients. These tests are becoming the

Pharmacogenomic tests bring a new level of precision to pharmaceu

Lon Castle, M.D., Senior Director, Personalized Medicine, Medco Health Solutions, Inc.

9:35 Medco Personalized Medicine: Advancing Healthcare

will remind participants that in the end, it was the tortoise that won

and use. This talk will focus on potential reasons for slow uptake of

alterations, or have generated test designs that are driven primarily by

one disease, where the multiple patents cover either alterations in one

gene or several genes involved in the disease. Such thickets have al

ready resulted in a failure to offer commercial tests for all possible gene

alterations, or have generated test designs that are driven primarily by

IP concerns. This talk will discuss possible solutions to the problem,

including the use of patent pools driven by medical standards.

5:20 Expert Panel: Bioscience Patent Law

• How do IP fragmentation and patent pools affect the clinical labs and end

users?

• How do they affect companies that own IP and commercialize IVDs?

• How do they affect companies and academic that discover new markers?

• Why are some licensing models slowing down the advancement of molecular
diagnostics?

5:50 Close of Day

FRIDAY, FEBRUARY 5

U.S. UNIVERSAL HEALTH - BREAKING NEWS

8:30 AM Chairperson's Opening Remarks

Brian T. Edmonds, Ph.D., Research Advisor, Global External Research & Development,

Lilly Corporate Center

8:35 Regulatory Considerations for Companion Diagnostics

and Personalized Medicine

Elizabeth Mansfield, Ph.D., Senior Genomics Advisor, Office of the Chief Scientist;

Director, Personalized Medicine, Office of In Vitro Diagnostic Device Evaluation and

Safety, Food & Drug Administration

Advances in genomics-based discovery and therapeutic agent targeting

have led to greatly increased interest in development of diagnostic/thera-
pneutic combinations that promise to deliver “personalized” therapy to

patients. With this vision comes the realization of the importance of

the diagnostic test performance upon which the therapeutic safety and

efficacy will rest. This presentation of regulatory issues for companion

diagnostic devices and codevelopment will address proposed regulatory

pathways for the diagnostic device, and emphasize the need for ade-
quate analytical and clinical validation.

9:05 Value Based Laboratory Tests -- What Went Wrong on the

Way to the Fair?

Ian S. Millett, Ph.D., RAC, Senior Consultant, Medical Devices, Biologics Consulting

Group, Inc.

In spite of tremendous interest, expenditures, and pro-active work

by industry, academia, and government, the personalized health care

revolution seems to have stalled in the area of new test development

and use. This talk will focus on potential reasons for slow uptake of

new diagnostic technology, will survey the upcoming landscape, and

will remind participants that in the end, it was the tortoise that won

the race.

9:35 Medco Personalized Medicine: Advancing Healthcare

Lon Castle, M.D., Senior Director, Personalized Medicine, Medco Health Solutions, Inc.

Pharmacogenomic tests bring a new level of precision to pharmaceu-
tical care, enabling treatment that is targeted to the unique genetic

characteristics of individual patients. These tests are becoming the

standard of care in many therapeutic areas, as physicians and payers

become more conversant with the value of testing. Medco's Personal-

ized Medicine healthcare can improve outcomes and reduce the over-

all costs of care.

10:05 Innovations in Molecular Diagnostics

and Sample Preparation Methods: Accelerating Sample-to-Result Diagnostics

Kevin Banks, Ph.D., Head of Marketing and Sales, Akonni Biosystems

Akonni Biosystems (Frederick, MD) was founded in 2003 and has over

20 patents with 13 others pending. The company’s core platform utilizes

gel-drop array technology optimized for developing medical applica-
tions, with an emphasis on greatly accelerating the time from sample

to result. This session will provide an introduction and overview of the

technology and platform. Preliminary results with a number of clinical

applications will be discussed.

10:20 Coffee Break

11:00 Economics of Having Diagnostics Reimbursed and the

Practical Challenges in Getting Reimbursement

Philip C.M. Ma, Ph.D., Director, McKinsey & Company, Inc.

The impact of diagnostics in influencing care continues to grow with

technology advancements in clinical genomics and other molecular

markers. In spite of this, the current reimbursement system in the U.S.
does not appropriate incentives for effective use - both over and under-

use of diagnostics can result. This talk will review how mis-aligned in-
centives can result from the under-lying micro-economics of different

stakeholders (physicians, patients, payors, and diagnostic manufactur-
ers), and will suggest a few ways to improve the micro-economic situ-

ation.

11:30 Panel Discussion: Personalized Medicine and

Challenges for Implementation

• How will universal healthcare impact your business?

• How will it impact molecular diagnostics adoption?

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or

Lunch on Your Own

1:00 Chairperson's Remarks

VALUE OF CONSUMER BASED GENOMICS: WHAT IS THE CONSUMER GOING TO DO WITH IT?

• How good is the information delivered by these tests?

• How well can people understand the results?

• How effectively can they help people manage their health?

1:05 Consumers and Their Genomes

Brian Vaughton, Ph.D., Founding Scientist, 23andMe

Over 30,000 individuals now have access to their personal genetic in-

formation through 23andMe’s web-based services. Consumers sign up for

these services to learn about their disease risk or carrier status, to

discover their ancestral roots, to find new relatives, or to participate

in research on a particular disease such as Parkinson's. This presenta-

tion will discuss the ongoing studies that are beginning to reveal how

people respond to their personal genetic information.

1:35 Talk Title to be Announced

Patrick F. Terry, CEO, Technic Solutions, LLC; Acting CEO, Grand Therapeutics, Inc.

2:05 Drinking from the Fire Hose: Are Consumers Ready?

Sharon Terry, MA, President and CEO, Genetic Alliance

2:35 Panel Discussion and Q&A with Audience

3:05 Close of Conference
3:50 Presentation Sponsored by Proteome Sciences plc, UK

Isotopic Mass Tags for the Facilitated Development of Multiplex SRM Mass Spectrometric Assays for Protein and Peptide Biomarkers
Helen Byers, Ph.D., Principal Research Scientist, Proteome Sciences plc, UK

Fit-for-purpose assays are essential for biomarker qualification. Selected Reaction Monitoring (SRM) is increasingly used for the quantitation of peptides and proteins, but is limited by expense and delay connected with synthesis of isotope-doped standard peptides and the difficulty to synthesize more complex standards (e.g. with post-translational modifications). Proteome Sciences has developed isotopic versions of its proprietary tandem mass tag (TMT) reagents to differently label sample and standard allowing to establish TMT-SRM, a method that allows the use of synthetic or natural reference standards to establish assays for any peptide or protein in any given sample material.

- Criteria

4:20 Reception in the Exhibit Hall (Sponsorship Available)

5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

Personalized Medicine: Commercial Hurdles to Adoption in an Era of Evidence Based Medicine
Moderator: Katherine Tynan Ph.D., Business Development & Strategic Consulting for Diagnostics Companies, Tynan Consulting LLC

- The evidence required for each of the stakeholders: analytical performance and clinical validity for the FDA, clinical utility for physicians, and medical necessity for payers.
- The strategic importance of “intended use statements” in guiding product/ test development
- The opportunities and challenges with reimbursement

Development and Application of Assays in the 3D Format
Moderator: Ray Mattingly, Ph.D., Associate Professor, Pharmacology, Wayne State University

- Challenges in standardization and feasibility of 3D cultures
- Development of 3D format for high-throughput assays
- Protocols for drug screening in 3D culture format
- Advanced 3D co-culture approaches to model tissues

How Innovative Technologies Are Selected, Evaluated, and Translated for Application in Diagnostics for Personalized Medicine to Enhance or Replace Conventional Diagnostics
Moderator: Kewal K. Jain, M.D., Professor, Jain PharmaBiotech

- The ideal molecular diagnostics laboratory for personalized medicine
- Selection, evaluation, and translation of new diagnostic technologies for personalized medicine
- Role of sequencing
- Future prospects of diagnostics for personalized medicine: supplementing, enhancing or replacing conventional diagnostics

Biomarkers of Efficacy
Moderator: Tracey Calpitts, Ph.D., Business Development, Companion Products, Abbott Molecular

- How does mechanism of action hypotheses translate to population science?
- When and how do we gather prevalence data?
- What priorities should we be making?

Next Generation Sequencing in the Clinical Diagnostics Laboratory
Moderator: Karl V. Voelkerding, M.D., Associate Professor of Pathology, University of Utah and Medical Director for Advanced Technology, ARUP Laboratories

- What are the first diagnostic applications that next generation sequencing will be used for?
- What improvements would facilitate translation of the technology into the clinical laboratory?
- How will laboratories process and interpret the large amounts of data generated?

6:20 Close of Day

THURSDAY, FEBRUARY 4

PERSONALIZING THERAPY: SERUM BIOMARKERS

8:25 AM Chairperson’s Remarks
Josip Blonder, M.D., Sr Research Scientist, Head, Quantitative Proteomics, NCI, Frederick

8:30 Personalized Oncoproteomics for Cancer Biomarker Discovery: Application to Renal Cell Carcinoma
Josip Blonder, M.D., Sr. Research Scientist, Head, Quantitative Proteomics, NCI

Discovery of diagnostic, therapeutic and prognostic markers is central to personalized treatment of cancer. Thus, proteomic approaches capable of characterizing the patient’s tumor phenotype using clinically relevant specimens are critically needed. A method that relies on tissue-directed oncoproteomics is described and applied for cancer biomarker discovery in the plasma of a patient diagnosed with renal cell carcinoma.

9:00 Protein Quantification Through Targeted Mass Spectrometry: The Way Out of Biomarker Purgatory?
Steven A. Carr, Ph.D., Director, Proteomics, The Broad Institute of MIT and Harvard

Immunoassays are widely used to measure protein biomarkers in patient blood, but useful antibody reagents do not exist for the vast majority of proteins. We are addressing this serious barrier by developing targeted assay methods employing mass spectrometry to screen and quantify low abundance proteins in plasma. This presentation will focus on the latest developments and applications of these technologies.

9:30 Mass Spec for Prostate Biomarkers, Assessing Aggressive vs. Non-Aggressive Prostate Cancer
Jianfeng Xu, Ph.D., Professor, Epidemiology, Prevention and Cancer Biology, Director, Ctr for Cancer Genomics, Wake Forest University School of Medicine

Three types of prostate cancer related genetic variants have been found from genome-wide association studies, including those associated with overall prostate cancer risk, aggressive prostate cancer risk, and higher baseline PSA levels. These genetic variants may have potential clinical utility. However, further studies are needed to assess their clinical validity and clinical utility.

10:00 A Novel Tool for Non-Invasive Disease Detection
Jack Leonard, Ph.D., Vice President of Technology Commercialization, febit Inc.

We developed a novel non-invasive diagnostic assay based on microRNAs. Our Biomarker Signature assay has shown an outstanding performance for the integrative detection of a broad panel of diseases and is well suited for high sample throughput at low cost since for each test less than one minute hands-on time is required. Moreover, our approach stands-out by high reproducibility and sensitivity while test-to-test variations are minimal.

10:15 Metabolite Profiling: Opportunities for Identification and Validation of Novel Biomarkers
Hajo Schiewe, Ph.D., Senior Manager, Business Development, Metanomics Health

Metabolite profiling is the parallel measurement of a broad range of endogenous and xenobiotic metabolites in a given biological sample. The metabolome reflects internal or external influences on the pathophysiology of an organism including drug treatment and disease status. Metanomics Health uses mass spectroscopy based metabolite profiling to identify and validate novel metabolite biomarkers for a range of applications in preclinical and clinical drug development, disease diagnostics and progression. The analysis and interpretation of metabolite changes can increase the mechanistic understanding of diseases, drugs and other influences on an organism.

Sponsored by febit
PERSONALIZING THERAPY: TISSUE BIOMARKERS

2:15 Chairperson’s Remarks
Linda McAllister, M.D., Ph.D.

2:20 Relating Biomarkers to Efficacy: The Efficacy Curve
Tracey Colpitts, Ph.D., Manager, Abbott Molecular
A method of predicting response in a subgroup defined by a biomarker will be discussed and demonstrated using data from therapeutic trials involving EGFR inhibitors in lung, colon, and breast cancer. Biomarkers that aid in selecting subgroups of patients of response were analyzed and compared. Striking similarities between the different cancers, therapies, and subgroups reveals a relationship between biomarkers and efficacy, which is visualized in the efficacy curve.

2:50 Network and Pathway Analysis of a Novel 3D Breast Carcinoma Model by Both Digital Gene Expression (DGE) and Whole Genome Array Analyses
Ray Mattingly, Ph.D., Associate Professor, Pharmacology, Wayne State University
We have developed a tractable, in vitro model of ductal carcinoma in situ (DCIS) based on 3D overlay culture in reconstituted basement membrane (rBM). We have applied and cross-validated whole genome microarray (Affymetrix) and digital gene expression (DGE) analyses (Illumina/Solexa) to explore the networks and pathways that underlie DCIS. DGE analysis revealed a broad range of products that are transcribed outside of standard (NCBI 36.3) genes models. These transcripts suggest truncations and changes in anti-sense driven regulatory pathways in DCIS.

3:20 Population Based in vivo Biomarker Discovery Using Engineered Human Tumors
Min Wu, Ph.D., Principal Scientist, Translational Research, AVEO Pharmaceuticals, Inc.
Human tumor populations exhibit significant inter-tumor variation, where each tumor harbors a unique set of genetic alterations that impact prognosis and response to treatment. Unfortunately, this variation results in low response rates in the clinic and creates significant challenges for drugs to meet regulatory endpoints. Cancer cell line based xenografts have traditionally been the preclinical model of choice to assess the efficacy of clinical compounds, however, such models exhibit inherent artifacts due to long term in vitro culture, and are unable to adequately capture natural variation seen in human tumor populations. To address this challenge, we have created a population based tumor model system based on Human-in-Mouse tissue transgenic human tumors that feature naturally occurring tumor variation akin to that observed in human tumor populations. Each tumor of the population has been comprehensively characterized at the RNA and DNA level, and the population has been adapted to conduct quantitative efficacy studies of anti-cancer agents and combinations, enabling correlations between response and the genetic context of the tumors. This platform enables us to identify and validate biomarkers of therapeutic response in an in vivo human tumor system.

2:10 Oil presentation and Morning Coffee
8:00 Plenary Keynote Session (See Page 26 for Details)
11:00 Chairperson’s Remarks
Harry Glorikian, Managing Partner, Scientia Advisors

11:05 Molecular Diagnostics as a Value Driver of Pharma/Pharma as a Value Driver for Molecular Diagnostics
Michael C. Little, Global Head, Diagnostics Development, Novartis Molecular Diagnostics
Molecular Diagnostics is a critical success factor for the future of pharmaceuticals and an essential aspect of the move toward personalized medicine. At the same time, to progress better healthcare and patient outcomes, it is imperative that the pharma industry’s understanding of both targeted drug discovery and drug commercialization is fully leveraged to enable innovative diagnostics to be put into clinical practice and influence physician decision-making. The keynote will focus on these elements and discuss 1-2 case studies of how we at Novartis are using our discovery and development approach to move towards bringing innovative companion and stand-alone diagnostic tests to market.

11:40 Building a Successful Diagnostics Business Model in the Era of Personalized Medicine
Richard Ding, CCO, bioMerieux Company
Personalized medicine has been generally accepted as an inevitable trend in healthcare. However, much debate is still ongoing related to a sustainable business model for diagnostics companies in this new space. This presentation will identify various challenges, risks and potential returns for diagnostic companies, explore partnership models and propose some basic framework to seize the growth opportunity of personalized medicine.

12:15 PM Personalized Medicine: It Takes a Village
Mark Stevenson, President & CEO, Life Technologies Corp.
New technologies, such as next generation sequencing, can be rapidly adopted in the research labs and help breakthroughs in our understanding of disease mechanisms for personalized medicine. But the journey from research technology to diagnostic systems is challenging and slow. As our understanding of disease increases the promise of personalized medicine is coming closer but what will it take to cross the bridge from research tool to routine diagnostics in personalized medicine. The presentation will focus on the journey Life Technologies has embarked on and the partnerships and collaborations necessary to translate the tools for the research lab into solutions personalized medicine.

12:50 Luncheon Presentation
Information Trends in Biomarker Research
Colin Williams, Ph.D., Director, Product Strategy, Thomson Reuters Healthcare and Science
In recent years, the quantity of data published on biomarker research has exploded. The challenge faced by researchers is to find vital, relevant information on the best biomarker quickly and reliably. In this discussion we will introduce BIOMARKERcenter, a comprehensive, fully-indexed biomarker information resource, and through case studies show how it aids the discovery process.

1:45 Dessert in the Exhibit Hall
Prostate cancer is the second most common cause of cancer-related death in men in the United States and afflicts one out of nine of those over the age of 65. There is an urgent need to develop biomarkers that can supplement PSA and increase its specificity for prostate cancer. The advent of high throughput profiling strategies has allowed scientists to look at global changes in genome, proteome and metabolome. Metabolomics, unlike genomics and proteomics, is a young science that has the potential to radically alter the future of healthcare, drug discovery, and drug delivery. It is the single best window into the cellular state discovered to date. Like the other omics-style sciences, where genomics is best understood as defining the genetic potential, transcriptomics is a window into the future (desired) direction of the cellular activity, and proteomics is a window to the functional potential of the cell; metabolomics, the omics science of metabolism, is the only window into the current and actual state of the cell (or by extension, organism) at a specific point in time.

Recently we have profiled the metabolome in prostate cancer progression using a combination of GC and LC chromatography. Our study quantified the levels of >1000 metabolites across 250 biospecimens. Results of the profiling study revealed elevated levels of sarcosine or N-methyl glycine to be associated with advanced prostate cancer. Importantly components of sarcosine pathway were found to regulate prostate cancer aggressivity. In addition to sarcosine we have defined additional metabolites that are being characterized in the context of prostate cancer progression. Our long term objective is to define a multiplex panel of metabolomic markers for prostate cancer progression.

Circulating tumor cells (CTC) are very rare and consist of about 1 in 108 or 109 cells in blood drawn from some patients with metastatic cancer. Enumeration of CTCTs has been shown to have prognostic value for patients with metastatic breast prostate and colorectal cancer, and is being evaluated to determine if a treatment-related reduction in CTC counts is predictive of therapeutic response. Comprehensive characterization (DNA, RNA and protein) of CTCs will significantly add to the value of CTC enumeration tests, and enable serial monitoring of CTCs for molecular changes occurring during disease progression and as a response to therapy. This presentation will review new approaches for the isolation and characterization of CTCs, and discuss how CTC-derived biomarkers will become a critical factor in the development of personalized treatment strategies in oncology.

High-throughput sequencing platforms provide an approach for detecting rare HIV-1 variants and documenting more fully quasispecies diversity. We applied this technology to understand viral dynamics at the sequence level, and deep sequencing provided a detailed view of the rapid evolutionary impact of selection.

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During the past several years it has become clear that alterations in the expression of microRNA genes contribute to the pathogenesis of most, perhaps all, human malignancies. These alterations can be caused by a variety of mechanisms, including deletions, amplifications, or mutations involving microRNA loci, by epigenetic silencing or by dysregulation of transcription factors targeting specific microRNAs. Since malignant cells show dependence on the dysregulated expression of microRNA genes, which in turn control or are controlled by dysregulation of multiple protein coding oncogenes or tumor suppressor genes, these small RNAs provide important opportunities for development of future microRNA based therapies.

9:05 microRNA Polymorphisms and the Future of Personalized Medicine
Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, National Cancer Institute, NIH

Referred to as the micromangers of gene expression, microRNAs are evolutionarily conserved small non-coding RNAs. Polymorphisms in the microRNA pathway can influence gene regulation and are emerging as powerful tools to study the biology of diseases. Detection of microRNA-polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

9:30 Living in a Sequen-omics World: Data Integration Issues and Challenges
David Sugarbaker, M.D., Chief, Thoracic Surgery, Brigham and Women’s Hospital

DNA sequencing and other “-omics” platforms (e.g., miRNA, microRNA, CGH, exon, SNP, and other arrays) have experienced a technological revolution in throughput and scale over the preceding decade that shows no sign of slowing. However, data storage and processing advances have outpaced the ability to fully integrate the resulting massive quantities of data into biologically meaningful and predictive models to apply to risk estimation, prevention, and cure of human diseases, most notably cancer. In addition, further technological innovation will continue to drive down costs which will exacerbate the problem in the near-term but over the long-term will bring more resources to bear in solving these issues and challenges. This session will provide a comprehensive view of the sequen-omics landscape and identify the key issues that will need to be addressed in the future for these platforms to positively affect human health.

We performed the first miRNAome-wide evaluation of specific miRNA expression in dried, forensically relevant biological fluids (blood, semen, saliva, vaginal secretions and menstrual blood). A panel of nine differentially expressed miRNAs was identified that permit the identification of the body fluid using 50pg of total RNA. miRNA profiling provides a promising alternative approach to body fluid identification for forensic casework.

10:05 10:05 LNA™ based Universal RT PCR System. A new Generation High Throughput QPCR Platform Optimized for Development microRNA based Molecular Diagnostic Assays on Clinical FFPE and Blood Serum and Plasma
Jacob Ulrik Fog, Ph.D., Scientific Manager, Diagnostic Product Development Division, Exiqon A/S

Usi ng a Locked Nucleic Acid (LNA™) based miRNA detection technology we have developed a high throughput QPCR system for detection of miRNAs in clinical paraffin-embedded tissue as well as blood derived plasma or serum. The use of the LNA™ bases adds critical specificity and sensitivity creating a more robust system for more rapid assay development in the clinical and diagnostic assay development.

10:20 Coffee Break

11:00 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers
Joanne B. Weidhaas, Ph.D., Assistant Professor, Therapeutic Radiology, Yale University

Since microRNAs are global regulators, small aberrations such as SNPs that disrupt their coding sequences or their target binding sites can alter cellular homeostasis and enhance cancer risk. MicroRNA binding site polymorphisms have turned out to be some of the strongest biomarkers of cancer risk, can act as biomarkers of outcome, and may be future targets for therapy.

11:30 Role of microRNA Based Profiling in Determining Tissue of Origin for Carcinoma of Unknown Primary
Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center

Carcinoma of unknown primary (CUP) is a heterogeneous disease where a patient presents with metastases without an identifiable primary. As more effective cytotoxic and targeted therapies emerge for additional known cancers, accurate identification of CUP subtypes will become increasingly important to the appropriate care of these patients. MicroRNA expression profiling is an emerging tool to help with identification of tissue of origin in patients with CUP.

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

INNOVATIVE DIAGNOSTICS FOR PERSONALIZED MEDICINE

1:00 Chairperson’s Remarks
Kewal K. Jain, M.D., Professor, CEO, Jain PharmaBiotech

1:05 Introduction to the Technologies and their Significance/Relevance for Personalized Medicine
Kewal K. Jain, M.D., Professor, CEO, Jain PharmaBiotech

1:35 A Low Cost Instrument for Microbead-Based Quantitative End-Point PCR and DNA Sequencing
Vera Garfinkel, Ph.D., Associate Professor, Stony Brook University

We present a novel, low cost instrument which performs microbead-based quantitative end-point PCR and CE based DNA sequencing. The instrument employs ultra fast, single photon sensitive detection of fluorescent signals in capillaries and operates as a module of the hardware/software suite GENOMETRICA - a novel, universal technology platform for molecular biology and medicine.

2:05 CNV Studies in Autism and other Neurological Disorders
Jim Chinitz, Chief Executive Officer, Population Diagnostics, Inc.

Historically, patients having a common complex disease have been lumped together and considered homogeneous according to phenotype. Population Diagnostics (“PDx”) has led a paradigm shift where appreciation is gaining for the heterogeneity of common disease which is likely caused by highly penetrant rare variants which are multi-locus and independently capable of generating the common phenotype. It is necessary to dissect phenotypes into genotypic differences to understand common disease and to personalize medicine. Beyond SNPs, there is a surprising abundance of structural variation in the genome called Copy Number Variants (CNVs), much of it occurring de novo. Recent studies have revealed “causative” rare CNV associations in autism, schizophrenia and ALS. In these models, the metrics that define the level of clinical relevance (i.e. odds ratios) of the rare variants is unprecedented, making them ideal candidates as novel biomarkers for predictive tests and beacons for molecular pathways. PDx is discovering and using a new standard of “causative” biomarkers and is paving the way for a next generation of diagnostic, personalized medicine and drug discovery applications.

2:35 Blood-based Diagnostics of Brain Injuries
Uwe R. Müller, Ph.D., VRT Product Development, Banyan Biomarkers, Inc.

Currently no FDA cleared lab tests exist for TBI and the diagnosis is based on complicated and expensive neurological and radio-imaging tests. Banyan has developed novel biomarkers for detection of TBI in the blood of patients within 2 hours of injury. We will present the results of our ongoing clinical studies, and our progress in the development of appropriate assay systems.

3:05 Close of Conference
We have made use of expression profiling to develop signatures of oncogenic pathway deregulation that can then be used to profile the state of these pathways within populations of tumors. In addition, the pathway signatures also link the patterns of pathway activation with therapeutics since we have shown that predicting the activation of a pathway also predicts sensitivity to drugs that target the pathway. We have extended this concept to develop more refined signatures that can dissect the complexities of many of the known signaling pathways, providing a more precise capacity to probe the activity or deregulation of the pathway and linking to a broader array of therapeutic agents.

12:10 PM Panel: Impact of Personalized Medicine on Oncology Drugs and Treatment
Additional Panelist: Mike Boswood, President, CEO, Thomson Reuters

12:40 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Dessert in the Exhibit Hall

PERSONALIZING THERAPY: TISSUE BIOMARKERS

2:15 Chairperson’s Remarks
Tracey Colpitts, Ph.D., Manager, Abbott Molecular

2:20 Relating Biomarkers to Efficacy: The Efficacy Curve
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A method of predicting response in a subgroup defined by a biomarker will be discussed and demonstrated using data from therapeutic trials involving EGFR inhibitors in lung, colon, and breast cancer. Biomarkers that aid in selecting subgroups of patients of response were analyzed and compared. Striking similarities between the different cancers, therapies, and subgroups reveals a relationship between biomarkers and efficacy, which is visualized in the efficacy curve.

2:50 Network and Pathway Analysis of a Novel 3D Breast Carcinoma Model by Both Digital Gene Expression (DGE) and Whole Genome Array Analyses
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3:00 Personalizing Medicine: It’s a System-Based Challenge
Franklyn G. Prendergast, M.D., Ph.D., Professor, Pharmacology, Biochemistry & Molecular Biology, Director, Center for Personalized Medicine, Mayo Clinic

3:10 Genomic Strategies for Personalized Cancer Treatment
Joseph R. Nevins, Ph.D., Barbara Levine Professor, Duke University
We have developed a tractable, 3D human tumor model system based on Human-in-Mouse engineered human tumors that feature naturally occurring tumor variation akin to that observed in human tumor populations. Each tumor of the population has been comprehensively characterized at the RNA and DNA level, and the population has been adapted to conduct quantitative efficacy studies of anti-cancer agents and combinations, enabling correlations between response and the genetic context of the tumors. This platform enables us to identify and validate biomarkers of therapeutic response in an in vivo human tumor system.

3:30 BREAK-OUT DISCUSSIONS in the Exhibit Hall

What is the Forecast for Epigenetics and microRNA?
Moderator: Enal Razvi, Ph.D., System Biosciences SBI
• Status of the microRNA and epigenetics market
• The research market for microRNA and epigenetics: growth and evolution
• Diagnostics and therapeutics development based on microRNA and epigenetic signatures
• Current challenges and opportunities in these spaces

Challenges to Whole Genome Sequencing
Moderator: s Ng, Ph.D., Assistant Professor, Genomic Medicine, J Craig Venter Institute
• Challenges to whole-genome sequencing
• Identifying de novo and re-current mutations in cancer
• Addressing tumor heterogeneity
• How can we move from characterizing gene variation to utilizing the whole genome
• Sequencing tumors rather than tumor cell lines
• The Complex genomic structure of tumor cells: de novo assembly or strategy to detect structural variants

Are there Cancers of Unknown Primary Tumors?
Moderator: Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.
• Debate over cancers of unknown primary tumors (CUP)
• Methods to detect CUPs
• Consequences of detection of primary

Gene Signatures in Cancer Diagnostics
Co-Moderators: Gary Geiss, Ph.D., Principal Scientist, NanoString Technologies and David Kern, MBA, Director, MyRaQa
• Developing a gene signature
• Validation of gene signatures
• Regulatory considerations for gene signature diagnostics

Systems Chemical Biology-A New Paradigm
Moderator: Ally Perlina, Senior Application Scientist, GeneGo Inc.
• Utilizing tools for drug repositioning
• Understanding side effects
• Understanding the mechanisms of action for drugs
• Networkable compounds
THURSDAY, FEBRUARY 4

PERSONALIZING THERAPY: SERUM BIOMARKERS

8:25 AM Chairperson’s Remarks
Josip Blonder, M.D., Sr Research Scientist; Head, Quantitative Proteomics, NCI Frederick

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10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

11:20 Metabolic Analysis of Prostate Cancer Progression
Arun Seeckumaran, Ph.D., Molecular Oncology Program, Medical College of Georgia

Prostate cancer is the second most common cause of cancer-related death in men in the United States and afflicts one out of nine of those over the age of 65. There is an urgent need to develop biomarkers that can supplement PSA and increase its specificity for prostate cancer. The advent of high throughput profiling strategies has allowed scientists to look at global changes in genome, proteome and metabolome. Metabo-lomics, unlike genomics and proteomics, is a young science that has the potential to radically alter the future of healthcare, drug discovery, and drug delivery. It is the single best window into the cellular state discovered to date. Like the other omics style sciences, where genomics is best understood as defining the genetic potential, transcriptomics is a window into the future (desired) direction of the cellular activity, and proteomics is a window to the functional potential of the cell; metabolomics, the omics science of metabolism, is the only window into the current and actual state of the cell (or by extension, organism) at a specific point in time.

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11:45 Circulating Tumor Cells: From Enumeration to Comprehensive Characterization
Nicholas C. Dracopoli, Ph.D., VP, Biomarkers, Centocor R&D, Inc., Johnson & Johnson

Circulating tumor cells (CTC) are very rare and consist of about 1 in 108 or 109 cells in blood drawn from some patients with metastatic cancer. Enumeration of CTCs has been shown to have prognostic value for patients with metastatic breast prostate and colorectal cancer, and is being evaluated to determine if a treatment-related reduc-tion in CTC counts is predictive of therapeutic response. Comprehensive characterization (DNA, RNA and protein) of CTCs will significantly add to the value of CTC enum-eration tests, and enable serial monitoring of CTCs for molecular changes occurring during disease progression and as a response to therapy. This presentation will review new approaches for the isolation and characterization of CTCs, and discuss how CTC-derived biomarkers will become a critical factor in the development of personalized treatment strategies in oncology.

12:10 PM Progress in Noninvasive Detection of Nucleic Acid Biomarkers
Charles R. Cantor, Ph.D., CSO, Sequenom, Inc.

Procedures have been developed to enhance the collection of RNA and DNA frag-ments that enter the peripheral circulation as a result of apoptosis. These include optimized methods of recovering small fragments, amplifying them and then detect-ing and quantifying sequence characteristics by nucleic acid mass spectrometry. The methods show promise in noninvasive prenatal diagnostics, tumor detection and characterization, and infectious disease agent identification. The overall process is considerably more sensitive and precise than commonly used alternatives.

12:35 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own
1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction

2:25 Plenary Keynote Presentation (See Page 26 for Details)

3:05 Refreshment Break in the Exhibit Hall

NEXTGEN SEQUENCING AS A CLINICAL TOOL

3:45 Chairperson’s Remarks

3:50 Enabling Personalized Medicine: The Growing Role of Next Generation Sequencing
German Pihan, M.D., Department of Pathology, Beth Israel Deaconess Medical Center

Ready access to the genome sequence of a patient is arguably the single most impor-tant factor in the implementation of personalized medicine. The recent development of massive parallel sequencing technologies promise to make personalized medicine soon a reality. Here I review the technological state-of-the-art as well as the clinical areas where massive parallel sequencing may have the greatest and most immediate impact.

4:20 Keynote Presentation

HLA Typing by High Resolution Technology
Henry A. Erlich, Ph.D., VP, Discovery Research, Human Genetics, Roche Molecular Systems, Inc.

4:50 Next Generation Sequencing for Hypertrophic Cardiomyopathy Diagnostics
Karl V. Voelkerding, M.D., Associate Professor of Pathology, University of Utah and Medical Director for Advanced Technology, ARUP Laboratories

Hypertrophic cardiomyopathy is an autosomal dominant disorder of cardiac sarco-mere structure and function leading to multiple cardiac conditions. At least 16 genes with over 450 mutations have been implicated in HCM. We have currently developed and tested a next generation sequencing approach for the analysis of this multi-gene disorder and are refining our approach for diagnostic application.
8:30 AM Chairperson’s Opening Remarks
Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

8:35 microRNA Polymorphisms and the Future of Personalized Medicine
Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, NCI, NIH

9:05 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers
Joanne B. Weidhaas, Ph.D., Assistant Professor, Yale University

9:30 Living in a Sequen-omics World: Data Integration Issues and Challenges
David Sugarbaker, M.D., Chief, Thoracic Surgery, Brigham and Women’s Hospital

10:05 ABC Transporters’ Role in Cancer Stem Cell Drug Resistance
Muhammad Al-Hajj, Ph.D., Director, Stem Cell Discovery Unit, GlaxoSmithKline

10:20 Coffee Break

11:00 Role of microRNA Based Profiling in Determining Tissue of Origin for Carcinoma of Unknown Primary
Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center

11:30 Keynote Presentation
Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

12:00 PM Luncheon Presentation
Available) or Lunch on Your Own

TARGETING CANCER STEM CELLS
1:00 Chairperson’s Remarks

1:05 Impact of Antibodies on Cancer Stem Cells: Discovering Underlying Pathways Essential to Cancer Stem Cell Biology
Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.

1:35 Understanding Tumor Cell Heterogeneity in NSCLC: Contributions to Resistance and Relapse
Erica L. Jackson, Ph.D., Scientist, Genentech, Inc.

2:05 New Visions of Cancer Therapy through the Prism of the Cancer Stem Cell Hypothesis
Justin O. Luthra, Ph.D., Research Associate, Department of Stem Cell Biology and Regenerative Medicine, Lerner Research Institute, Cleveland Clinic Foundation

2:30 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers
Joanne B. Weidhaas, Ph.D., Assistant Professor, Yale University

5:20 HIV Dynamics Taught by Sequencing
Ramy Arnaout, M.D., DPhil, Associate Director, Clinical Microbiology, BIDMC Staff Pathologist, Department of Pathology, BIDMC and Harvard Medical School, Beth Israel Deaconess Medical Center

High-throughput sequencing platforms provide an approach for detecting rare HIV-1 variants and documenting more fully quasispecies diversity. We applied this technology to understand viral dynamics at the sequence level associated with antiviral treatment failure. Failure was associated with extreme, rapid shifts in population frequencies toward specific resistant forms, and deep sequencing provided a detailed view of the rapid evolutionary impact of selection.

5:50 Close of Day

FRIDAY, FEBRUARY 5

MICRONRNA DIAGNOSTICS FOR CANCER: TRANSLATING INFORMATION TO PRACTICAL USE

8:30 AM Chairperson’s Opening Remarks
Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

8:35 Keynote Presentation
Causes and Consequences of microRNA Dysregulation in Cancer
Carla M. Croce, M.D., Professor, Internal Medicine, College of Medicine & Public Health, The Ohio State University

During the past several years it has become clear that alterations in the expression of microRNA genes contribute to the pathogenesis of most, perhaps all, human malignancies. These alterations can be caused by a variety of mechanisms, including deletions, amplifications or mutations involving microRNA loci, by epigenetic silencing or by dysregulation of transcription factors targeting specific microRNAs. Since malignant cells show dependence on the dysregulated expression of microRNA genes, which in turn control or are controlled by dysregulation of multiple protein-coding oncogenes or tumor suppressor genes, these small RNAs provide important opportunities for development of future microRNA based therapies.

9:05 microRNA Polymorphisms and the Future of Personalized Medicine
Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, NCI, NIH

Referred to as the micromanagers of gene expression, microRNAs are evolutionarily conserved small non-coding RNAs. Polymorphisms in the microRNA pathway can influence gene regulation and are emerging as powerful tools to study the biology of diseases. Detection of microRNA-polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

9:30 Living in a Sequen-omics World: Data Integration Issues and Challenges
David Sugarbaker, M.D., Chief, Thoracic Surgery, Brigham and Women’s Hospital

DNA sequencing and other “omics” platforms (e.g., mRNA, microRNA, CGH, exon, SNP, and other arrays) have experienced a technological revolution in throughput and scale over the preceding decade that shows no sign of slowing. However, data storage and processing advances have outpaced the ability to fully integrate the resulting massive quantities of data into biologically meaningful and predictive models to apply to risk estimation, prevention, and cure of human diseases, most notably cancer. In addition, further technological innovation will continue to drive down costs which will exacerbate the problem in the near-term but over the long-term will bring more resources to bear in solving these issues and challenges. This session will provide a comprehensive view of the sequen-omics landscape and identify the key issues that will need to be addressed in the future for these platforms to positively affect human health.

10:05 Sponsored Presentation
(Sponsorship Opportunity Available)

10:20 Coffee Break

11:00 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers
Joanne B. Weidhaas, Ph.D., Assistant Professor, Yale University

Since microRNAs are global regulators, small aberrations such as SNPs that disrupt their coding sequences or their target binding sites can alter cellular homeostasis and enhance cancer risk. microRNA binding site polymorphisms have turned out to be some of the strongest biomarkers of cancer risk, can act as biomarkers of outcome, and may be future targets for therapy.

11:30 Role of microRNA Based Profiling in Determining Tissue of Origin for Carcinoma of Unknown Primary
Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center

Carcinoma of unknown primary (CUP) is a heterogeneous disease where a patient presents with metastases without an identifiable primary. As more effective cytotoxic and targeted therapies emerge for additional known cancers, accurate identification of CUP subtypes will become increasingly important to the appropriate care of these patients. microRNA expression profiling is an emerging tool to help with identification of tissue of origin in patients with CUP.

12:00 PM Luncheon Presentation
(Sponsorship Opportunity Available) or Lunch on Your Own

TARGETING CANCER STEM CELLS
1:00 Chairperson’s Remarks

1:05 Impact of Antibodies on Cancer Stem Cells: Discovering Underlying Pathways Essential to Cancer Stem Cell Biology
Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.

Cancer stem cells are thought to mediate tumor initiation, metastasis, and recurrence. We have isolated and characterized CSCs from a variety of major tumor types and have found that these cells are preferentially resistant to many current therapies. As part of our effort to develop novel agents targeting CSCs, we have developed an anti-DLL4 antibody that blocks Notch signaling. Anti-DLL4 inhibits tumor growth through multiple mechanisms including a reduction in CSC frequency.

1:35 Understanding Tumor Cell Heterogeneity in NSCLC: Contributions to Resistance and Relapse
Erica L. Jackson, Ph.D., Scientist, Genentech, Inc.

Tumors are made up of a heterogeneous mixture of cell types and it is possible that distinct cell populations play unique roles in tumorigenesis. We are studying functionally defined cell populations to determine what distinguishes chemo-resistant cells from bulk tumor cells.

2:05 ABC Transporters’ Role in Cancer Stem Cell Drug Resistance
Muhammad Al-Hajj, Ph.D., Director, Stem Cell Discovery Unit, GlaxoSmithKline

One of the mechanisms by which residual disease become chemo-resistant is via the decreased efficiency of chemo-therapeutics through the action of ATP-binding cassette (ABC) proteins that are variably expressed by the tumor cells and tend to be up-regulated in some cancer stem cells. The clinical relevance of the ABC transporters in the context of cancer stem cells is paramount and their application requires better understanding of the role individual transporters play in the mechanism and the development of more specific inhibitors with minimal off target effects. Here we’ll discuss the role of two specific transporters in pancreatic and colon cancer stem cells and their value as therapeutic targets.

2:35 New Visions of Cancer Therapy through the Prism of the Cancer Stem Cell Hypothesis
Justin O. Luthra, Ph.D., Research Associate, Department of Stem Cell Biology and Regenerative Medicine, Lerner Research Institute, Cleveland Clinic Foundation

The failure of conventional therapies to fundamentally alter the survival of advanced and metastatic cancers has many causes but one appears to be the striking cellular heterogeneity in most cancers. The cancer stem cell hypothesis posits that tumors contain a cellular hierarchy of differentiation and tumor propagation potential. As studies have demonstrated that cancer stem cells display therapeutic resistance, angiogenic potential, and a propensity towards invasion/metastasis, the identification of signaling pathways and molecular targets in cancer stem cells may yield improved cancer therapies.

3:05 Close of Conference
CASE STUDIES FROM CHEMISTRY TO THE CLINIC

Scott B. Hoyt, Ph.D., Research Fellow, Department of Basic Chemistry, Merck Research Laboratories Voltage-gated Cav2.2 calcium channels control the release of neurotransmitter at presynaptic terminals, and thus play a critical role in pain signaling. The state-independent Cav2.2 blocker ziconotide, a peptide that must be administered via intrathecal injection, has demonstrated clinical efficacy in the treatment of severe chronic pain. State-dependent Cav2.2 blockers may likewise provide clinical pain relief without adversely affecting other nerve functions.

Michael Henning, Ph.D., Vice Director & Head, Discovery Technologies, F. Hoffmann-La Roche Ltd

Contributions of Water Molecules to Ligand-Receptor Binding

Interpreting structure-activity data is often challenging even with the availability of crystal structures. The role of solvent thermodynamics in protein binding sites is often overlooked but can be important in explaining experimental data. Here, we present a statistical thermodynamic approach to the treatment of binding site water molecules and show that hydration site displacement patterns can be used to explain SAR trends, ligand selectivity, and site-directed mutagenesis. Applications of the method to the A2A adenosine receptor, PDZ domains, a broad range of kinases, and other systems of pharmaceutical interest will be discussed.

3:50 Thermodynamic
Sponsored by SCHRÖDINGER

Contributions of Water Molecules to Ligand-Receptor Binding
Christopher Higgs, Ph.D., MRSC, Senior Applications Scientist, Schrödinger, LLC

Gary W. Small, M.D., Parlow-Solomon Professor on Aging, Professor of Psychiatry & Biobehavioral Sciences, Director, UCLA Center on Aging

4:20 Reception in the Exhibit Hall (Sponsorship Available)
5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall
6:20 Close of Day

THURSDAY, FEBRUARY 4

FRAGMENT-INSPIRED AND STRUCTURE-GUIDED MEDICINAL CHEMISTRY

8:25 AM Chairperson’s Remarks
Charles Reynolds, Ph.D., Research Fellow, Computer Aided Drug Discovery, Johnson & Johnson

8:30 Drug Discovery Facilitated by Fragment Screening Efforts
Michael Henning, Ph.D., Vice Director & Head, Discovery Technologies, F. Hoffmann-La Roche Ltd

Rapid gain in potency of compounds by structure based drug design together with the high sensitivity of biophysical methods like Surface Plasmon Resonance (SPR) enable the use of fragment molecules to guide drug discovery efforts. The lecture will review the fragment screening efforts at Roche and analyze benefits and challenges of the approach from these experiences. Drug targets like β-secretase or chymase are used as case studies.

9:00 Synthesis, in vitro and in vivo Evaluation of PI3K Inhibitors
Matthew Burger, Ph.D., Research Investigator II, Global Discovery Chemistry, Novartis Institutes for Biomedical Research

Phosphoinositide-3-Kinase (PI3K) is an important oncology target due to the deregulation of its signaling pathway in a wide variety of human cancers. A lead series from a combinatorial library was identified that potently inhibits PI3K. Using SBDD the lead series was optimized to yield PI3K inhibitors with suitable PK properties to establish a PK/PD-efficacy relationship in a mouse A2780 xenograft model.
9:20 Design, Synthesis and Optimization 2-aminoquinazolines as PDK1 Inhibitors
Savithri Ramurthy, Ph.D., Research Investigator, GDC/ONC, Novartis Institutes of Biomedical Research, Emeryville, CA

Herein, we describe the use of iterative structure-guided design to discover two sets of leads from the series Quinazolines as PDK1 inhibitors. The in vitro and in vivo activity of potent PDK1 inhibitors will be discussed along with the medicinal chemistry approaches utilized to optimize the chemical series for kinase selectivity, efflux, and hERG.

9:40 Presentation Sponsored by www.evotec.com

Fragment Based Drug Discovery at Evotec - Application to the identification of BACE and PDE10a inhibitors
James Madden, Ph.D., Principal Scientist, Evotec (UK) Limited

Evotec’s FBDD platform (EVOlutionTM) integrates orthogonal screening technologies, namely; biochemical, NMR and SPR to test fragments in a high throughput, highly sensitive mode. Evotec has successfully applied this technology in a number of programs. This presentation will describe 2 case studies where EVOlutionTM has been used to discover BACE and PDE10a inhibitors.

10:00 Luncheon Presentation Sponsored by Thomson Reuters

The Role of Medicinal Chemistry in Translational Research
Josep Prous, Jr., Ph.D., MBA, Vice President and Chief Scientific Officer, Thomson Reuters Healthcare & Science

The biomedical community has embraced the translational research approach to finding better and safer medicines. However, to meet the promises of this approach, researchers need a knowledge-based methodology in which the constituent disciplines share data appropriately. This talk will show how medicinal chemistry provides a bridge between early biology findings and clinical application of new molecular entities.

10:30 Poster Competition refreshment Break & Raffles in the Exhibit Hall

Charles Reynolds, Ph.D., Research Fellow, Computer Aided Drug Discovery, Johnson & Johnson

Quantum methods are just beginning to find wider application in drug discovery. We have used pair-wise decomposition of protein-ligand interaction energies, computed using the DivCon program, to analyze the interactions that drive potency in a series of protein kinase B inhibitors. These computed interaction energies were used to derive two heat maps: (1) an interaction energy map and (2) an SAR map. These interaction energies, and resulting maps, provide detailed information not otherwise available for identifying the residues in an active site that are most critical for ligand binding.

12:00 PM The Emperor’s New Crystal: Examples of X-ray Blooper’s; a Cautionary Tale
Edward Kesicki, Ph.D., Director, Small Molecule Drug Discovery, Infectious Disease Research Institute

I will give examples of “solved” structures in which incorrect ligands were fitted to the electron density map of a kinase co-crystal, a result of a typographical error in the paperwork sent to the crystallographer. In addition, I will show a class of selective P13 kinase inhibitors that would have never been discovered using known X-ray crystal structures.

12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Ice Cream Refreshment Break in the Exhibit Hall
9:35 Biotransformation to Enable Chemistry SAR
Douglas Spracklin, Ph.D., Director, Pharmacokinetics, Dynamics and Metabolism, Pfizer, Inc.
Biotransformation science has evolved well beyond traditional structural elucidation of metabolites. Contemporary biotransformation data is especially well suited to aid chemistry SAR development, i.e., identifying metabolic hot spots, non-obvious metabolic pathways, potential reactive metabolites, etc. Knowledge around these attributes can be extremely helpful in prioritizing chemical series and selecting individual molecules for development.

10:05 Sponsored Presentation (Sponsorship Opportunity Available)

10:20 Coffee Break
IMAGING AS AN EXCITING TOOL IN DRUG DISCOVERY
Chair: Michael A. Letavic, Research Fellow, Neuroscience, Johnson & Johnson Pharmaceutical R&D

11:00 Imaging Drug Action in the Human Brain
Joanna Fowler, Ph.D., Senior Chemist, Brookhaven National Laboratory
Radiotracers and drug molecules labeled with short-lived positron emitting isotopes such as carbon-11 (t1/2: 20.4 min), fluorine-18 (t1/2: 110 min) or nitrogen-13 (t1/2: 10 min) are unique scientific tools for measuring biochemical transformations and drug pharmacokinetics and pharmacodynamics in the living human and animal body.

11:30 Image Analysis Considerations for Preclinical, in vivo Medical Imaging
Matt Silva, Head, Imaging Sciences, Millennium, The Takeda Oncology Company
With the expanding role of preclinical and translational imaging in drug research, it is necessary to consider not only study design and imaging modality but also visualization and image quantification. This presentation will review the role of imaging technologies and show examples of experiments and image analysis procedures, including kinetic analysis of dynamic contrast-enhanced MRI and bone topology analysis from 3D CT data.

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

TARGETS IN HOT PURSUIT II
1:00 Chairperson’s Remarks
Nick Terrett, Ph.D., Chief Scientific Officer, Ensemble Discovery Corp.

1:05 Modulators and Consequences of Hsp90 Regulation by Small Molecules
Brian S. J. Blagg, Ph.D., Associate Professor of Medicinal Chemistry, The University of Kansas; Winner of the 2009 David W. Robertson Award in Medicinal Chemistry
The 90 kDa heat shock proteins (Hsp90) are molecular chaperones required for the refolding of denatured proteins and the maturation of nascent polypeptides into their biologically active, three-dimensional structures. In fact, numerous proteins represented in all six hallmarks of cancer are dependent upon Hsp90 for conformational maturation. Innovative approaches toward C-terminal inhibition of Hsp90 will be discussed.

1:35 Design and Synthesis of RDEA119, a Potent and Orally Bioavailable MEK Inhibitor
Jean-Michel Vernier, Ph.D., VP, Chemistry Discovery, Ardea Biosciences
This presentation will discuss the design, synthesis and structural-activity relationship that led to the discovery of RDEA119, a novel highly potent and selective MEK inhibitor currently in Phase I clinical trial. RDEA119 is being developed under a global license agreement with Bayer HealthCare.

2:05 From a Concept towards a First-In-Class Drug for a Human Amyloid Disease
Jeffery W. Kelly, Ph.D., Chair, Molecular and Experimental Medicine, Lita Annenberg Hazen Professor of Chemistry, The Skaggs Institute, The Scripps Research Institute
The seminar will cover the twenty-one year adventure from our initial demonstration that rate-limiting transthyretin tetramer dissociation and monomer misfolding was sufficient for transthyretin amyloidogenesis linked to neurodegeneration, to the recent clinical trial results of FoldRx demonstrating that a transthyretin kinetic stabilizer halts neurodegeneration in familial amyloid polyneuropathy. This is the first pharmacologic evidence supporting the validity of the amyloid hypothesis.

2:35 DNA-Programmed Chemistry Approach to Macroyclic Lead Compounds
Nick Terrett, Ph.D., CSO, Ensemble Discovery Corp.
DNA-programmed chemistry is an integrated platform for the synthesis and screening of macrocycles that interact with protein-protein drug discovery targets such as the oncology target, BCL-XL. We have also discovered a series of macrocycles that competitively antagonize the interaction of TNFα with TNF receptors in both biochemical and cell-based assays, and that also have anti-inflammatory activity in vivo.

3:05 Close of Conference
INFORMATICS Channell

therapies. This session will introduce a strongly typed repository of linked investigators must describe and verify systems about the life science uni-
verse. To assist investigators, we will adopt an enterprise-wide scientific workflow platform enabling research informatics organizations to shift their efforts towards scientific innovation. Researchers apply scientific workflows for in silico experimentation and exploration, leading to scientific hypotheses and discoveries. Enterprise environments enable researchers to share and evolve their scientific workflows, further increasing research productivity. Examples of scientific workflows and the setting required to run scientific workflow platforms effectively in pharmaceutical research will be discussed.

11:00 Chairperson’s Remarks
David M. Sedlock, Ph.D., Senior Director, Research & Development Systems, Millennium, The Takeda Oncology Company

11:10 Enterprise Scientific Workflow Environment Drives Innovation
Daniel J. Chin, Ph.D., Senior Principal Research Scientist, Roche Palo Alto

Most informatics investments increase the efficiency of drug discovery. The introduction of an enterprise-wide scientific workflow platform enables research informatics organizations to shift their efforts towards scientific innovation. Researchers apply scientific workflows for in silico experimentation and exploration, leading to scientific hypotheses and discoveries. Enterprise environments enable researchers to share and evolve their scientific workflows, further increasing research productivity. Examples of scientific workflows and the setting required to run scientific workflow platforms effectively in pharmaceutical research will be discussed.

11:40 The Pistoia Alliance, Inc.—A Construct for Precompetitive Collaboration
Chris Waller, Ph.D., Senior Director, Precompetitive Collaborations, Research, Development & Medical Informatics, Worldwide Technology, Pfizer, Inc.
The Pistoia Alliance has been established to provide the foundation of data standards, ontologies and associated web-services to enable the Pharmaceutical discovery workflow through common business terms, relationships and processes. Current progress, learnings and how companies, academics and others can participate in this approach will be described.

12:10 PM Recent Strategies with Cloud, Wikis, Ontologies and Open Source Data Standards
Giles M. Day, Senior Director, BBC Informatics, Pfizer, Inc.

12:40 Luncheon Presentation I  Sponsored by Microsoft* By attending Microsoft’s Luncheon presentation you are opting to receive further communications from Microsoft.
Personalized Medicine: The Missing Pieces
Jim Karkanias, Senior Director of Applied Research & Technology, Microsoft Health Solutions Group
Discoveries to make personalized medicine a reality depend on leveraging the “open universe” of life sciences data. To assist investigators with ad hoc questions, hypothesis generation, and validation, investigators must describe and verify systems about the life science universe. This session will introduce a strongly typed repository of linked data that makes it possible to conceive and deliver game-changing therapies.

1:00 Chairperson’s Remarks
David M. Sedlock, Ph.D., Senior Director, Research & Development Systems, Millennium, The Takeda Oncology Company

1:10pm Luncheon Presentation II Sponsored by BioFortis
Empowering Scientists with Hypothesis-Driven Data Exploration
Jian Wang, Ph.D., CEO, BioFortis inc.
A significant bottleneck on productivity in translational research is the inability for scientists to directly interrogate data by themselves. We present a novel solution & case study to demonstrate how, with the right tools, scientists can be more self-sufficient, efficient and productive, while enabling informatics specialists to focus more on higher value contributions instead of mundane ad hoc data manipulations.

1:45 Dessert in the Exhibit Hall

2:15 Chairperson’s Remarks
David M. Sedlock, Ph.D., Senior Director, Research & Development Systems, Millennium, The Takeda Oncology Company

2:20 Integrated Informatics Systems for R&D
Vaibhav A. Narayan, Ph.D., Senior Director, Integrative Neurosciences & Biomarkers, Johnson & Johnson Pharmaceutical Research & Development

2:50 Executive Panel with Q&A
Are We Integrating the Right Data: Extending Beyond Laboratory Data to Decisions Impacting Project Success
Moderator: David M. Sedlock, Ph.D., Senior Director, Research & Development Systems, Millennium, The Takeda Oncology Company
• Data Aggregation vs. Data Integration
• Data Management vs. Knowledge Management
• Integrating Data Management Systems across Multiple Sites
• Effective Data Integration in Translational Medicine Research
• Role of Open Source Technology in Systems Design
• Is There a ‘Cloud’ in Your Future?
• Barriers Sharing Research and Development Data
• Process Management vs. Technology Considerations when Deploying New Systems
Panelists:
Susie Stephens, Director, Biomedical Informatics, Pharmaceutical Research & Development, Johnson & Johnson
Daniel J. Chin, Ph.D., Senior Principal Research Scientist, Roche Palo Alto
Chris Waller, Ph.D., Senior Director, Precompetitive Collaborations, Research, Development & Medical Informatics, Worldwide Technology, Pfizer, Inc.
Giles M. Day, Senior Director, BBC Informatics, Pfizer, Inc.

3:50 Presentation
Workflow based Enterprise Informatics
Frank Brown, Ph.D., Vice President & Chief Scientific Officer, Accelrys
Accelrys is producing a new generation of Enterprise Informatics systems for chemical, biological and image data registration and mining. The new generation features workflow driven application logic and business rules, clients that leverage the latest collaborative environments such as Microsoft SharePoint, and novel storage techniques to handle the complexity and diversity of today’s data types.

4:05 Sponsored Presentation (Sponsorship Opportunity Available)

4:20 Reception in the Exhibit Hall (Sponsorship Available)

5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall

6:20 Close of Day
DATA INFORMATION AND KNOWLEDGE MANAGEMENT

8:25 AM Chairperson’s Remarks
Thomas P. Hill, Principal, The Leverage Innovation Group; former Director, Learning and Knowledge Management, Genentech

8:30 SparkLab 360 - The Complete System for Managing your Lab Research
Sponsored by SparkLix Bio-IT
Roi Paz, Chief Executive Officer, SparkLix Bio-IT
Electronic lab notebooks (ELNs) and laboratory information management systems (LIMS) are essential tools in lab management. SparkLix – an Innovation Award recipient from the Association for Laboratory Automation – developed SparkLab 360, which integrates features from ELNs and LIMS in one user-friendly system. This presentation focuses on how SparkLab enables design, planning, execution and analysis of the entire research process.

9:00 Knowledge for Strategic Advantage: Accelerating the R&D Cycle
Sponsored by BIG
Thomas P. Hill, Principal, The Leverage Innovation Group; former Director, Learning and Knowledge Management, Genentech
This presentation focuses on key elements of knowledge leveraged for strategic advantage in the Life Sciences industry, the key challenge of how to accelerate the R&D process by using collaborative informatics technologies and an examination of specific scientific business solution implementations for results. In addition, the key features of a robust collaborative scientific business solution will be identified.

9:30 ASAP-Emphasizing Multidimensional Drug Discovery
Sponsored by ASAP
W. Patrick Walters, Ph.D., Senior Research Fellow & Group Head, Computational Drug Discovery Technologies, Vertex Pharmaceuticals, Inc.
ASAP is a new software platform designed to help drug discovery teams make better decisions. ASAP provides an intuitive overview of the data that also allows scientists to easily “drill down” and examine the details of particular experiments. A combination of “filters” and heat maps allows teams to focus on aspects of the data while remaining aware of the “big picture”.

10:00 RlSe Architecture – Architectural Aspects of an Integrated Research Informatics Platform
Sponsored by Relius
Ajay Shah, Ph.D., MBA, PMI, Director of Research Informatics, Elan Pharmaceuticals Inc.
Elan and Infosys are building a research data integration platform called RlSe (Research Informatics System at elan). RlSe enables registration of biological entities, their inventory, associated workflows, and integration with chemical data utilizing a workflow driven, multi-tiered, SOA based architecture built on the Microsoft.NET platform. To maximize extensibility in a research environment, the database combines Entity-Attribute-Value design for flexible definition of entities, efficiency prioritized OLTP schema for inventory management, and a planned ETL interface to a semantic database.

10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

11:30 Agile Software Development: Meeting the Rapidly Changing Needs in Drug Discovery
Sponsored by Infosys
Man-Ling Lee, Ph.D., Senior Program Analyst, Discovery Chemistry, Small Molecule Drug Discovery Technologies, Genentech, Inc.
To support drug discovery project teams meeting their timelines, the Com-pChem/ChemInformatics Group at Genentech has established an agile approach to satisfy the changing needs. The basis are two flexible software platforms, AEREA (Aestel Scientific Information) and Pipeline Pilot (Accelrys). The presentation will discuss the implementation and impact of two applications: one for lead selection and one for DMPK data analysis.

INTEGRATIVE DATA MANAGEMENT THROUGH CLOUD, WIKIS, ONTOLOGIES & SEMANTIC WEB

12:00 PM Application of Translational Informatics in Tailored Therapeutics
Sponsored by Oracle
Susie Stephens, Director, Biomedical Informatics, Pharmaceutical Research & Development, Johnson & Johnson

12:30 Managing Research Portfolios – Can IT help you?
Sponsored by Oracle
Arvind Balakrishnan, Vice President Life Sciences, Oracle
Joe Duncan, Chief Executive Officer, Tennado
Research departments work on hundreds of thousands of molecules, markers and targets. How can we simplify information gathering across your scientific community in order make consistent portfolio management decisions? This talk will focus on using Oracle technologies like semantic web; and how portfolios can be reflected in best in class portfolio management tools.

1:00 Luncheon Presentation
Sponsored by HP
SOA-based IT Framework for Life Science Research
David A. Medina, Worldwide Life Science and Pharma Segment Executive, Hewlett-Packard Company
This presentation will present a collaborative platform for bioinformatics used in bioresearch based on a scalable, standards-based, easy-to-deploy, SOA-based architecture. This platform will facilitate the integration of intra-organizational research efforts and enable inter-organizational R&D collaboration. The platform will also enable pharma R&D organizations to effectively access disparate data sources and facilitate the cross-analysis of genomic, proteomic and clinical data.

1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction

2:25 Plenary Keynote Presentation (See Page 26 for Details)

3:05 Refreshment Break in the Exhibit Hall

INTEGRATIVE DATA MANAGEMENT THROUGH CLOUD, WIKIS, ONTOLOGIES & SEMANTIC WEB CONT.

3:45 Chairperson’s Remarks
Sponsored by Oracle
Susie Stephens, Director, Biomedical Informatics, Pharmaceutical Research & Development, Johnson & Johnson

3:50 Data Integration—What’s in it for Me?
Sponsored by Elan
Randal Chen, Ph.D., Director, Research Informatics, Amgen, South San Francisco

4:20 Semantic Web and Cloud Computing for Integrative Data Management and Analysis Infrastructure
Sponsored by Elan
Jonas S. Almeida, Ph.D., Abeil-Hanger Distinguished Professor, Bioinformatics and Computational Biology, University of Texas, M.D. Anderson Cancer Center
The systems nature of biological processes and the scalability of cloud computing created an irresistible trend towards distribution of both the data and the ecosystem of applications that analyze them. Accordingly, at M.D. Anderson Cancer Center we are exploring the use of semantic web to render distributed infrastructure manageable and its contents safely discoverable. An open-source prototype was developed, see s3db.org.

4:50 Collaborative Drug Discovery Humanitarian and Commercial Researcher Network Case Studies
Sponsored by Elan
Barry A. Bunin, Ph.D., Chief Executive Officer & President, Collaborative Drug Discovery (CDD), Inc.
Collaborative Drug Discovery (CDD) has created a community based platform that combines traditional drug discovery informatics with Web2.0 features to provide the best of both worlds. Recent efforts to selectively arrest TB in the dormant phase working with leading researchers and mining SAR data will be presented. A global community of leading TB researchers supported by leading foundations will be reviewed. Advances from communities working together on commercial
drug discovery bringing together industry, foundations, and academia will also be emphasized.

5:05 Panel: Drug Discovery Collaborations in 2010
Moderator: Barry A. Bunin, Ph.D., Chief Executive Officer & President, Collaborative Drug Discovery (CDD), Inc.
- Biopharmaceutical – CRO collaborations
- Virtual Pharmaceutical collaborations
- PPP (academic-industry-foundation) collaborations
Panelists:
Vaihava A. Narayan, Ph.D., Senior Director, Integrative Neurosciences & Biomarkers, Johnson & Johnson Pharmaceutical Research & Development
Uli Schmitz, Ph.D., Director, Structural Chemistry, Gilead
Adam Renslo, Ph.D., Associate Director, Chemistry, Adjunct Assistant Professor, Pharmaceutical Chemistry

5:50 Close of Day

FRIDAY, FEBRUARY 5

TRANSLATIONAL INFORMATICS—HOW FAR HAVE WE COME?

8:30 AM Chairperson's Opening Remarks
Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.

8:35 Implementing a Translational Biomarker Strategy to Reduce Attrition in Drug Development
Irina Antonijevic, M.D., Ph.D., Director, Translational Research, Biological Research, Lundbeck Research, Inc., USA
Early efforts towards the discovery of molecular biomarkers for CNS disorders are encouraging. However, confirmation, and ultimately validation of such biomarkers is dependent on state-of-the-art bioinformatics analyses as well as assay development. These prerequisites will ensure identification of biomarkers that are reproducible and hence of clinical relevance.

9:05 High Content Mining of Disease Biomarkers
Jake Chen, Ph.D., Assistant Professor, Informatics & Computer Science, Indiana University School of Informatics; Director, Indiana Center for Systems Biology and Personalized Medicine, Indiana University-Purdue University Indianapolis; Founder, MedeoLinx, Inc.
To facilitate the interpretation of raw Omics data into detailed disease-specific knowledge of candidate biomarkers, we developed a “high-content biomarker mining” software system. The system can help manage and correlate molecular functions, molecular connectivity, biological pathways, and literature information. Its application into the current biomarker development process will help improve the success rate and quality of candidate biomarkers.

Eric Schadt, Ph.D., Chief Scientific Officer, Pacific Biosciences
While there has been an explosion of technologies that enable more comprehensive characterizations of complex biological processes like common human diseases, we are still unable to glimpse a large enough fraction of the biology of these systems to build models that are predictive enough to achieve clinical utility. However, with a new wave of technologies on the horizon, providing for the capability to examine the activity of single molecules real time, we will soon be capable of generating the right scale and diversity of data (DNA sequence, RNA sequence, real time monitoring of mRNA translation, full characterizations of base modifications in genomes and transcriptomes) at low cost to dramatically enhance the construction of models for common human diseases that achieve clinical utility. I will cover the single molecule real time (SMRT) technologies from Pacific Biosciences and how these technologies will revolutionize our ability to characterize living systems, and then present a number of integrative biology approaches to taking the types of data SMRT technologies will generate to get at predictive models of disease that can be used to drive the identification and validation of drug targets and biomarkers.

10:05 Sponsored Presentation (Sponsorship Opportunity Available)

10:20 Coffee Break

11:00 Profiling Patients to Drive Biomarker Development
N. R. Nirmala, Ph.D., Director, Biomarker Analysis and Informatics Unit, Translational Sciences, Novartis Institutes of Biomedical Research
Gene expression profiling is one of the key ways in which a genome-wide view of a patient’s response to drug treatment can be obtained. Such a molecular level view can provide strategies for customized therapies in many contexts. In this talk, the opportunities and challenges that this technology presents will be discussed with a couple of case studies. Extension of this approach to other technologies will also be presented in the context of biomarker development.

11:30 Panel: Informatics at R&D Interphases
Moderator: Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.
- Linking clinical outcome with molecular data: filling the gaps
- Capturing uniform clinical language for outcomes
- Compatible and user-friendly data systems—can one size fit all?
- Disease cohorts-how many, how big, what is acceptable quality

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

INTEGRATED GENOMIC, BIOLOGY, AND IMAGE DATA

1:00 Chairperson's Remarks
Ajay Shah, Ph.D., MBA, PMP, Director, Research Informatics, Elan Pharmaceuticals, Inc.

1:05 Image Analysis Considerations for Pre-clinical, in vivo Medical Imaging
Matt Shue, Head, Imaging Sciences, Millennium, The Takeda Oncology Company
With the expanding role of preclinical and translational imaging in drug research, it is necessary to consider not only study design and imaging modality but also visualization and image quantification. This presentation will review the role of imaging technologies and show examples of experiments and image analysis procedures, including kinetic analysis of dynamic contrast-enhanced MRI and bone topology analysis from 3D CT data.

1:35 RISe Prowler: A Semantic Web Approach to Integrating External and In-house Biology and Chemistry Information
Ajay Shah, Ph.D., MBA, PMP, Director, Research Informatics, Elan Pharmaceuticals, Inc.

2:05 Development of a Registration System for Biomarkers in a Collaborative Special Interest Group
Jeremy Packer, Ph.D., Head, Bioinformatics, Abbott

2:35 Development of Combination Therapies for Multiple Sclerosis Using Systems Level Informatics
Frederic S. Young, Ph.D., Chief Scientist, Vicus Therapeutics
We start with a multilevel systems physiology model that combines metabolomic analysis with integrated physiological analysis. The model is used to define a set of systems informative features of ontogeny, phylogeny, homeostasis, and repair that distinguishes the disease state from homeostasis. We describe our use of this systems informatic signature as an algorithm for the development of combination therapies for multiple sclerosis.

3:05 Close of Conference

INTEGRATED GENOMIC, BIOLOGY, AND IMAGE DATA
CANCER & INFORMATICS Channel

sure. Genes required for cell enrichment or cell depletion can then be
or depleted in this mixed population in response to a selective pres-
approaches requiring many multi-well plates, screens using lentiviral
fective and less labor-intensive manner. Unlike arrayed shRNA library
low for high-throughput screening of the entire genome in a cost-ef-

1:10-1:40 A Novel Genome-Wide Screening
Application Using Pooled Viral miRNA-adapted
shRNA (shRNAmiR) Libraries
Kodie Jensen Spagy, Ph.D., Research Scientist, Thermo Fisher Scientific
Pooled shRNA libraries are powerful genetic discovery tools that al-
low for high-throughput screening of the entire genome in a cost-
effect and less labor-intensive manner. Unlike arrayed shRNA library
approaches requiring many multi-well plates, screens using lentiviral
shRNA pools can be performed in a single tissue culture plate. Clonal
populations of cells expressing an individual shRNA become enriched
or depleted in this mixed population in response to a selective pres-
ure required for cell enrichment or cell depletion can then be
deconvoluted by next-generation sequencing or microarrays hybrid-
ized with barcode sequences corresponding to each shRNA in the pool.
Here, we present a novel pooled shRNA screening approach for iden-tyfying regulators of endogenous gene expression. Epithelial cell adhe-
sion molecule (EpCAM), a cell surface receptor that is highly expressed
in a variety of tumorogenic cells, promotes cell proliferation and tumor
formation via transcriptional activation of mitogenic genes. Thus, Ep-
CAM represents a target for the development of new cancer therapeu-
tics. We performed a whole-genome pooled shRNA screen to identify
novel regulators of EpCAM expression. OVCA8 cells were transduced
with the thermo Fisher Scientific Decode™ RNAi Viral shRNAmir Pools.
Following puromycin selection, we used magnetic-activated cell sort-
ing (MACS) to separate cells on the basis of EpCAM protein expression.
Genomic DNA was isolated from EpCAM+ and EpCAM- cells and the
shRNAs enriched or depleted within each population were identified
using custom microarrays. The genes targeted by shRNAs enriched in
EpCAM- cells were identified as candidate regulators of EpCAM expres-
sion. This work demonstrates that pooled shRNA libraries may be used
in a variety of novel screening strategies, including the identification of
novel regulators of tumor-associated genes.

1:45 Dessert in the Exhibit Hall
1:45 Dessert in the Exhibit Hall

WORKING BACKWARDS IN CANCER: FROM THE CLINIC TO DISCOVERY
2:15 Chairperson’s Remarks
Michael Liebman, Ph.D., Managing Director, Strategic Medicine, Inc.

2:20 Using Drug-Induced Feedback Loops to Identify Indications
and Combination Partners
Donald Bergstrom, Director, Experimental Medicine Oncology, Merck
James W. Watters, Associate Director, Molecular Profiling Oncology, Merck

Treatment with molecular targeted agents can result in compensatory feedback regula-
tion as cells respond to inhibition of signaling pathways. We will present clinical evi-
dence that treatment with a small molecule inhibitor of gamma secretase results in
pathway modulation and compensatory feedback, and describe pre-clinical experiments
designed to leverage this concept for drug response prediction.

2:50 Genomic Solutions to Diagnostic and Prognostic Clinical
Predictions in Head and Neck Cancer
Geoffrey Childs, Ph.D., Professor of Pathology, Albert Einstein College of
Medicine
Richard V. Smith, M.D., FACS, Professor, Vice-Chair, Department of
Otorhinolaryngology-Head and Neck Surgery, Montefiore Medical
Center and Albert Einstein College of Medicine

The strategy our group employs is to utilize the data obtained from high throughput as-
says including gene expression measurements of mRNA and miRNA, global methylation
patterns of DNA and global proteomics to develop prognostic and diagnostic signatures
to predict outcome, local regional recurrence presence/absence of lymph node metas-
tasis at initial diagnosis and to predict optimal treatment options.

3:20 Moving Research Closer to the Bedside, in vitro and in vivo
Analyses with Primary Tumors
Fred Poordad, M.D., Chief of Hepatology, Liver Disease and Transplant
Center, Cedars-Sinai Medical Center, Xin Wei Wang, Ph.D., Senior
Investigator Head, Liver Carcinogenesis Section, Laboratory of Human
Carcinogenesis, National Cancer Institute, NIH, and Michael R. Briggs,
Ph.D., Senior Director, Biology, Vertex Pharmaceuticals, Inc.

The incidence of Primary Liver Cancer is increasing in the west and constitutes a tremen-
dous burden on world health as the third leading cause of cancer deaths worldwide.
The 5 year survival rate is a dismal 11 %, due in large part to late diagnosis and limited
treatment options. The etiology of this devastating disease as well as current and pro-
posed new therapies will be discussed. Steps to better diagnose and stratify patients
targeted therapy will be considered as a new and exciting phase of cancer research.
Finally, a move towards more relevant research will be presented as an hypothesis that
Clinical Outcomes?

and other diseases will be described. A gap from molecular pathway to clinical outcome in a single step. Case studies in cancer unbiased indication discovery and AE profiling, and it is unique. It helps to bridge the MoA of 8,000 indications and 12,000 adverse events (AEs). This is simultaneous, mechanism of action (MoA). Biovista screens the MoA of any drug or target against the MoA of 8,000 indications and 12,000 adverse events (AEs). This is simultaneous, mechanism of action (MoA). Biovista screens the MoA of any drug or target against

Challenges to Whole Genome Sequencing

Moderator: s Ng, Ph.D., Assistant Professor, Genomic Medicine, J Craig Venter Institute

• Challenges to whole-genome sequencing
• Identifying de novo and re-current mutations in cancer
• Addressing tumor heterogeneity
• How can we move from characterizing gene variation to utilizing the whole genome
• Sequencing tumors rather than tumor cell lines
• The Complex genomic structure of tumor cells: de novo assembly or strategy to detect structural variants

Are there Cancers of Unknown Primary Tumors?

Moderator: Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

• Debate over cancers of unknown primary tumors (CUP)
• Methods to detect CUPs
• Consequences of detection of primary

Gene Signatures in Cancer Diagnostics

Co-Moderators: Gary Geiss, Ph.D., Principal Scientist, NanoString Technologies and David Kern, MBA, Director, MyRoQA

• Developing a gene signature
• Validation of gene signatures
• Regulatory considerations for gene signature diagnostics

Systems Chemical Biology-A New Paradigm

Moderator: Ally Perlina, Senior Application Scientist, GeneGo Inc.

• Utilizing tools for drug repositioning
• Understanding side effects
• Understanding the mechanisms of action for drugs
• Networkable compounds

6:20 Close of Day

THURSDAY, FEBRUARY 4

REAL EXAMPLES OF INTEGRATING PATHWAY DATA

8:25 AM Chairperson’s Remarks

Megan Laurance, Ph.D., Senior Scientist, Ingenuity Systems, Inc.

8:30 Keynote Presentation

Kenneth H. Buetow, Ph.D., Associate Director, Bioinformatics and Information Technology, National Cancer Institute

9:00 Cooperative and Complementary Genetic Selection in Brain Tumors

Markus Bredel, M.D., Ph.D., Director, Northwestern Brain Tumor Institute Research Program, Assistant Professor, Department of Neurological Surgery, Robert H. Lurie Comprehensive Cancer Center, Northwestern University Feinberg School of Medicine, and Lurie Center for Cancer Genetics Research and Center for Genetic Medicine

Brain tumors are a disease of the genome. These tumors show recurrent patterns of genetic aberrations. Dissecting which genetic events function cooperatively to deregulate principal signaling pathways in brain tumors and which are complementary to such deregulation will help developing refined therapeutic strategies to treat these complex diseases.

9:30 Expression Based Patient Stratification for Cancer Prognostics

Peter I. van der Spek, Ph.D., Department of Bioinformatics, Erasmus MC - Medical Faculty

Systems biology approaches in life sciences and health open new perspectives for patient stratification. Microarray and next-generation sequencing techniques provide vast volumes of data and detailed information about natural variants vs. mutations in the underlying molecular etiology of the disease. Knowledge bases allow scientists to place their research results in perspective.

10:00 Functional Analysis of Omics Data in Cancer

Yuri Nikolaevsky, Ph.D., CEO, GeneGo, Inc.

High-throughput assays are indispensable in studies of complex human diseases. Numerous methods have been developed for Omics data analysis. I will describe GeneGo techniques of pathway, network, and interaction analysis, and summarize recent results of our collaborative studies on breast, colorectal, pancreatic, and glial cancers. I will also describe functional analysis of predictive gene signatures developed for FDA’s MAQC project.

10:15 Cellular Target Profiling and Quantitative Phosphoproteomics Reveal Insight into a Drug’s Efficiency and Cellular Mode of Action

Jutta Fritz, Ph.D., Head of Technology, KINAXO Biotechnologies

System-wide approaches integrating drug target identification and global phosphoproteomics depict a compound’s cellular mode of action and its impact on signal transduction. KINAXO’s chemical proteomics and global quantitative phosphoproteomics platform revealed Sorafenib’s target profile and allowed quantification of phosphorylation patterns in relation to drug administration, thereby facilitating monitoring of the integration of signaling and pointing at additional therapeutic applications.

10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

11:30 microRNA Expression Profiling for the Identification of Forensically Relevant Biological Fluids

Jack Ballantyne, Ph.D., Professor, Department of Chemistry, Associate Director for Research, National Center for Forensic Science, University of Central Florida

We performed the first miRNAome-wide evaluation of specific miRNA expression in dried, forensically relevant biological fluids (blood, semen, saliva, vaginal secretions and menstrual blood). A panel of nine differentially expressed miRNAs was identified that permit the identification of the body fluid using 50pg of total RNA. miRNA profiling provides a promising alternative approach to body fluid identification for forensic casework.

12:00 pm Gene Expression Signatures of Pathway Activity as Biomarkers in Oncology: RAS Pathway Signature

Andrey P. Loboda, Ph.D., Research Fellow, Oncology Molecular Profiling, Merck Research Laboratories

12:30 Luncheon Presentation I

Overview of Metabolomics

John Rylas, Ph.D., Chief Executive Officer, Metabolon, Inc.

Metabolomics is the global profiling of biochemicals and metabolites in biological samples and provides a snapshot of the metabolic state of a biological system. As such, it can rapidly characterize and identify metabolic changes caused by drugs, disease, diet or environment effects. This talk provides an overview of metabolomics and the technology requirements for profiling hundreds of biochemicals. The technology platform deployed at Metabolon involves the separation of analytes on three independent analytical platforms (GC-MS, LC-MS/MS/+), LC-MS/MS/-(+)). Proprietary software processes the mass spectral data and retention times by matching the run data to a database of biochemical standards. This “chemo-centric” approach results in the positive identification of hundreds of biochemicals in a single sample. Through statistical analysis, the significant changes are identified and mapped onto biochemical pathways. Because biochemicals are closely related to biological phenotype, identification of affected pathways not only provides insight into the biological mechanism but uncovers biomarkers useful in diagnosing and monitoring.
High-throughput gene expression analysis techniques generate large amounts of data, which pose a particular challenge of transforming expression data into meaningful hypotheses for target discovery and candidate biomarker identification. Traditional approaches for interpreting expression data rely on mapping differentially expressed genes to canonical pathways, biological processes, or disease states. However, understanding the transcriptional regulators and upstream signaling events that lead to differential gene expression can help better identify the molecular mechanisms that influence changes in gene expression profiles, facilitating discovery of targets which themselves are not differentially expressed, but which are key to underlying disease mechanisms. In this session we present a case study in which ExPlain™, a tool that employs the BIOBASE Knowledge Library™ for promoter and regulatory network analysis, was used to uncover target and biomarker candidates from a cancer gene expression experiment, by identifying upstream signaling molecules likely involved in the underlying pathways leading to the cancer disease state.

1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction

2:25 Plenary Keynote Presentation (See Page 26 for Details)

3:05 Refreshment Break in the Exhibit Hall

SYSTEMS BASED APPROACHES TO CANCER SEQUENCING: PUTTING TOGETHER A NETWORK OF CHANGES

3:45 Chairperson’s Remarks

Robert L. Strausberg, Ph.D., Deputy Director, J. Craig Venter Institute

3:50 Whole Genome Sequencing in Cancer

Gad Getz, Ph.D., Head, Cancer Genome Analysis, The Broad Institute

4:20 Systematic Discovery of Cancer Gene Fusions using Paired End Transcriptome Sequencing

Chandan Kumar, Ph.D., Michigan Center for Translational Pathology, University of Michigan

Gene fusions represent common genetic aberrations in cancers that can serve as specific biomarkers and therapeutic targets. The recent discovery of recurrent gene fusions in prostate and lung cancers portends similar aberrations in other common carcinomas. We employ paired end transcriptome sequencing and customized bioinformatic pipelines to characterize gene fusions and chimeric transcripts in cancer.

4:50 Mapping Cancer Genomics Data to Pathways

David Haussler, Ph.D., Professor & Director, Biomolecular Science & Engineering, University of California, Santa Cruz

It is essential, but challenging to interpret cancer genomics data in terms of biological meaningful perturbations of molecular pathways within tumor cells. I will discuss a new Cancer Genomics Browser, on the web at genome-cancer.ucsc.edu, that accomplishes this through large-scale data analysis and probabilistic modeling. This methodology is currently being used in several large-scale cancer studies, including the ISPY breast cancer trial, the TCGA project and by one of the SU2C Dream Teams.

5:20 Analyzing Coding Variants

Pauline Ng, Ph.D., Senior Scientist, Human Genomic Medicine, J. Craig Venter Institute

Whole genome and whole exome sequencing are identifying a large number of coding variants. Some of these coding variants may have functional consequences that lead to disease. I will discuss the behavior of coding variants and the webtools we have made to analyze them.

5:50 Close of Day

FRIDAY, FEBRUARY 5

MICRORNA DIAGNOSTICS FOR CANCER: TRANSLATING INFORMATION TO PRACTICAL USE

8:30 AM Chairperson’s Opening Remarks

Dalia Cohen, Ph.D., Chief Scientific Officer, Rosetta Genomics, Inc.

8:35 Keynote Presentation: Causes and Consequences of microRNA Dysregulation in Cancer

Carlo M. Croce, M.D., Professor, Internal Medicine, College of Medicine & Public Health, The Ohio State University

During the past several years it has become clear that alterations in the expression of microRNA genes contribute to the pathogenesis of most, perhaps all, human malignancies. These alterations can be caused by a variety of mechanisms, including deletions, amplifications or mutations involving microRNA loci, by epigenetic silencing or by dysregulation of transcription factors targeting specific microRNAs. Since malignant cells show dependence on the dysregulated expression of microRNA genes, which in turn control or are controlled by dysregulation of multiple protein coding oncogenes or tumor suppressor genes, these small RNAs provide important opportunities for development of future microRNA based therapies.

9:05 microRNA Polymorphisms and the Future of Personalized Medicine

Prasun J. Mishra, Ph.D., Laboratory of Cancer Biology & Genetics, National Cancer Institute, NIH

Referred to as the micromanagers of gene expression, microRNAs are evolutionarily conserved small non-coding RNAs. Polymorphisms in the microRNA pathway can influence gene regulation and are emerging as powerful tools to study the biology of diseases. Detection of microRNA-polymorphisms can potentially improve diagnosis, treatment and prognosis in patients and has profound implications in the fields of pharmacogenomics and personalized medicine.

9:35 Living in a Sequen-omics World: Data Integration Issues and Challenges

Gavin Gordon, Ph.D. Co-Director, Thoracic Surgery Oncology Lab, Brigham & Womens Hospital

DNA sequencing and other “-omics” platforms (e.g., mRNA, microRNA, CGH, exon, SNP, and other arrays) have experienced a technological revolution in throughput and scale over the preceding decade that shows no sign of slowing. However, data storage and processing advances have outpaced the ability to fully integrate the resulting massive quantities of data into biologically meaningful and predictive models to apply to risk estimation, prevention, and cure of human diseases, most notably cancer. In addition, further technological innovation will continue to drive down costs which will exacerbate the problem in the near-term but over the long-term will bring more resources to bear in solving these issues and challenges. This session will provide a comprehensive view of the sequen-omics landscape and identify the key issues that will need to be addressed in the future for these platforms to positively affect human health.

10:05 Sponsored Presentation (Sponsorship Opportunity Available)

10:20 Coffee Break

11:00 The Onco-SNP and Cancer Risk: microRNA Binding Site Polymorphisms as Biomarkers

Joanne B. Weidhaas, Ph.D., Assistant Professor, Therapeutic Radiology, Yale University

Since microRNAs are global regulators, small aberrations such as SNPs that disrupt their coding sequences or their target binding sites can alter cellular homeostasis and may be future targets for therapy.

11:30 Role of microRNA Based Profiling in Determining Tissue of Origin for Carcinoma of Unknown Primary

Gauri Varadhachary, M.D., Associate Professor of Medicine, Department of G.I. Medical Oncology, M.D. Anderson Cancer Center

Carcinoma of unknown primary (CUP) is a heterogeneous disease where a patient presents with metastases without an identifiable primary. As more effective cytotoxic and targeted therapies emerge for additional known cancers, accurate identification of CUP subtypes will become increasingly important to the appropriate care of these patients. MicroRNA expression profiling is an emerging tool to help with identification of tissue of origin in patients with CUP.

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own
TARGETING CANCER STEM CELLS

1:00 Chairperson’s Remarks
Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.

1:05 Impact of Antibodies on Cancer Stem Cells: Discovering Underlying Pathways Essential to Cancer Stem Cell Biology
Tim Hoey, Ph.D., VP, Cancer Biology, OncoMed Pharmaceuticals, Inc.

Cancer stem cells are thought to mediate tumor initiation, metastasis, and recurrence. We have isolated and characterized CSCs from a variety of major tumor types and have found that these cells are preferentially resistant to many current therapies. As part of our effort to develop novel agents targeting CSCs, we have developed an anti-DLL4 antibody that blocks Notch signaling. Anti-DLL4 inhibits tumor growth through multiple mechanisms including a reduction in CSC frequency.

1:35 Understanding Tumor Cell Heterogeneity in NSCLC: Contributions to Resistance and Relapse
Erica L. Jackson, Ph.D., Scientist, Department of Tumor Biology and Angiogenesis, Genentech, Inc.

Tumors are made up of a heterogeneous mixture of cell types and it is possible that distinct cell populations play unique roles in tumorigenesis. We are studying functionally defined cell populations to determine what distinguishes chemo-resistant cells from bulk tumor cells.

2:05 New Visions of Cancer Therapy through the Prism of the Cancer Stem Cell Hypothesis
Justin D. Lathia, Ph.D., Research Associate, Department of Stem Cell Biology and Regenerative Medicine, Lerner Research Institute, Cleveland Clinic Foundation

The failure of conventional therapies to fundamentally alter the survival of advanced and metastatic cancers has many causes but one appears to be the striking cellular heterogeneity in most cancers. The cancer stem cell hypothesis posits that tumors contain a cellular hierarchy of differentiation and tumor propagation potential. As studies have demonstrated that cancer stem cells display therapeutic resistance, angiogenic potential, and a propensity towards invasion/metastasis, the identification of signaling pathways and molecular targets in cancer stem cells may yield improved cancer therapies.

2:35 Close of Conference
BIOLOGICS Channel plans. safety will be discussed as will issues pertaining to manufacturing and clinical application will be in stable ischemic stroke patients. Models of efficacy and logeneically and implanted directly at the site of injury. Our first clinical ap-

SB623 cells are derived from bone marrow stromal cells (MSCs). They have Casey Case, Ph.D., Vice President of Research, SanBio, Inc.

Damien Bates, M.D., Ph.D., Chief Medical Officer, SanBio, Inc.

3:20 MSC-Derived SB623 Cells for Stable Stroke

Edward Wirth III, M.D., Ph.D., Medical Director, SanBio, Inc.

3:55 Featured Presentation

12:40 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Dessert in the Exhibit Hall

• CONSIDERATIONS FOR ADVANCING REGENERATIVE MEDICINE INTO THE CLINIC

2:15 Chairperson’s Remarks

Dawn Driscoll, MBA, Ph.D., Principal, DCi Biotech

2:20 hESC-Derived Oligodendrocyte Progenitor Cells-GRNOPC1 for Acute Spinal Cord Injury

Edward Wirth III, M.D., Ph.D., Medical Director, Geron Corporation

2:50 From Tissue Engineering to Regenerative Medicine: An Evolution in Understanding

Damien Bates, M.D., Ph.D., Chief Medical Officer, Organogenesis, Inc.

3:20 MSC-Derived SB623 Cells for Stable Stroke

Casey Case, Ph.D., Vice President of Research, SanBio, Inc.

SB623 cells are derived from bone marrow stromal cells (MSCs). They have shown great potential in models of CNS regeneration. The cells are used al-logeneically and implanted directly at the site of injury. Our first clinical application will be in stable ischemic stroke patients. Models of efficacy and safety will be discussed as will issues pertaining to manufacturing and clinical plans.
10:00 Presentation
Programmable, Fully Automated Microfluidic Stem Cell Culture System
Marc A. Unger, Ph.D., CSO, Fluidigm Corporation

Cell reprogramming techniques require treating cells with multiple factors, either for conversion of differentiated cells into induced pluripotent stem (iPS) cells or for conversion of pluripotent cells into a desired type of differentiated cells. Fluidigm is developing a versatile, automated cell culture system which can culture cells, carry out multi-factor dosing experiments, and image the cells in both fluorescence and incident light modes in any desired time sequence. The results of in-chip cell culture and multi-factor dosing experiments will be described and applications discussed.

10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

11:30 Integrated Chemical Genomics Reveals Modifiers of Cell Fate in Pluripotent Stem Cells
April Pyle, Ph.D., Assistant Professor, Microbiology, Immunology & Molecular Genetics, Eli and Edythe Broad Center of Regenerative Medicine & Stem Cell Research; Jonsson Comprehensive Cancer Center, University of California, Los Angeles

While hESCs can be maintained in vitro, cells grown in continuous culture have been shown to develop cytogenetic and genetic aberrations associated with cancer in vivo. Additionally, hESCs exhibit poor survival as single cells following dissociation, which limits the ability to perform genetic manipulation and homogenous differentiation of hESCs. In order to identify pathways involved in regulating self-renewal and survival without instability, we have developed a cell-based high content screening (HCS) assay using small molecules. This method provided a comprehensive approach for studying hESC fate in vitro and identified a number of novel regulators of hESC growth.

12:00 PM Biocompatible Grafted Carbon Nanotubes as Scaffolds for Preferential hESC Differentiation
Jennifer Lu, Ph.D., Professor, School of Engineering, University of California, Merced

Presented is our research on using biocompatible grafted carbon nanotubes as scaffolds for preferential neuron cells differentiation from hESCs. It has been found that carbon nanotube-based scaffolds promote the growth factor adsorption, leading to more selective differentiation. It has been observed that surface properties such as hydrophilicity and charge can play important roles in directing hESC differentiation. Novel responsive scaffolds have been synthesized and the potential use of such dynamic scaffolds for cell growth, differentiation and proliferation will be discussed.

12:30 Luncheon Presentation Sponsored by
Multiplex Biomarker Assays for Translational Research
Robert Umek, Ph.D., Director of Research, Meso Scale Discovery

1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction

2:25 Plenary Keynote Presentation (See Page 26 for Details)

3:05 Refreshment Break in the Exhibit Hall

IPS CELLS: FROM SCREENING TO THERAPY

Induced pluripotent stem cells (iPS) cells, the most recent advancement in stem cell research, even further widen and generate applications for stem cell research. IPS cells exhibit great promise in drug discovery and screening as well as in regenerative medicine. IPS Cells: From Screening to Therapies not only explores current methods of generating, maintaining, and utilizing IPS cells but will address the shift in using them to contribute to Shaping the Future of Regenerative Medicine.

3:45 Chairperson’s Remarks
Bruce Conklin, M.D., Senior Investigator, Gladstone Institute of Cardiovascular Disease, Professor of Medicine, Division of Medical Genetics, University of California, San Francisco

IPS CELLS FOR DISEASE MODELS

3:50 Featured Speaker

Potential Promise of IPS Cells for Understanding Disease Progression

3:20 IPS Cells for Cardiovascular Models and Diagnostics
Bruce Conklin, M.D., Senior Investigator, Gladstone Institute of Cardiovascular Disease, Professor of Medicine, Division of Medical Genetics, University of California, San Francisco
Our functional genomic experiments focus on GPCR signaling pathways in pluripo-
tent ES cell-derived cardiac myocytes. We use high-throughput gene inactivation methods, including siRNA and gene trapping in ES cells, and then analyze ES cell-derived cardiomyocytes. Our initial signaling studies focused on mouse ES cells, and ES cell-derived mice. We are using human IPS cells for similar signaling studies and to produce models of human cardiac disease including Long QT syndrome.

4:50 Using iPS Cells to Model Neurological Diseases
Clive N. Svendsen, Ph.D., Professor, Anatomy & Neurology, University of Wisconsin

5:20 Hoseok Song, Ph.D., Professor of Biology, University of California, San Diego

5:50 Close of Day

FRIDAY, FEBRUARY 5

IPS CELLS FOR REGENERATIVE HEALING

8:30 AM Chairperson’s Opening Remarks
Timothy J. Kamp, M.D., Ph.D., Professor of Medicine & Physiology; Co-Director Stem Cell and Regenerative Medicine Center, University of Wisconsin

8:35 MicroRNA-Target Gene Networks as Fundamental Factors in the Next Generation Regenerative Strategies
Preethi H. Gunaratne, Ph.D., Assistant Professor, Department of Biology & Biochemistry, University of Houston

MicroRNAs are small non-coding RNAs that integrate multiple genes within and across biological pathways. LIN28/let-7; c-MYC-E2F/miR-17-92 and Oct4/Sox2/miR-302-cyclin D1 networks have been tightly linked to embryonic (ES) and more recently to iPS cells. We have also uncovered additional miRNAs regulated by Ronin, a non-canonical pluripotency factor that target genes regulating cytoskeletal remodeling and epigenetic silencing. Discussed is the potential role of these key miRNAs in the next generation regenerative strategies.

9:05 IPS Cells Offer New Alternative and Early Treatment in Genetic Diseases
Yuet Wai Kan, MB, BS, D.Sc., Professor, Department of Medicine, University of California, San Francisco

Two alternatives are currently available to parents if the prenatal diagnosis of a serious genetic disease is made: to terminate the pregnancy, or to continue it and take care of a seriously ill child. Generation of IPS cells from the amniotic fluid or CVS cells used for the diagnosis, correction of the mutation, and differentiation of the cells into specific tissues may in the future offer a new alternative. In addition, it will allow early treatment of the genetic disease, an important consideration in diseases where organ damage begins early in life.

9:35 Pluripotent Stem Cells Derived from Adult Human Testes
Martin Dym, Ph.D., Professor, Biochemistry & Molecular & Cellular Biology, Georgetown University Medical Center

Male germline stem cells obtained from adult human testes can be reprogrammed spontaneously to generate pluripotent stem cells. The production of these “non-canonical” IPS cells is spontaneous, and do not require the addition of exogenous genes, some of which may be cancer causing. Our results suggest that human spermatogonial stem cells have great potential for cell-based, autologous organ regeneration therapy for various diseases and it is thus possible that in the near future men could be cured of disease with a biopsy of their own testes.

10:05 Poster Presentation: Differentiation of Human Embryonic and Human Induced Pluripotent Stem Cells Along the Otic Lineage
Kinuxa Masaki, Stanford University

10:20 Coffee Break

IPS CELLS FOR REGENERATIVE HEALING CONT’D

11:00 Directed Differentiation of Human IPS Cells Generates Active Motor Neurons
William Lowry, Ph.D., Assistant Professor, Department of Molecular, Cell and Developmental Biology, University of California, Los Angeles

A study of gene expression profiles of mouse and human ESCs and IPSCs suggests that, while IPSCs are quite similar to their embryonic counterparts, a recurrent gene expression signature appears in IPSCs regardless of their origin or the method by which they were generated. Shown is how both hESCs and hiPSCs can differentiate to form fully functional differentiated progeny. We are now setting out to understand whether the differentiated progeny of hESCs and hiPSCs share commonalities or differences as was observed in their undifferentiated parent cells in an attempt to make predictions about whether these two types of pluripotent cells have similar potentials in regenerative medicine.
11:30 Functional Cardiomyocytes Derived from Human Induced Pluripotent Stem Cells

Timothy J. Kamp, M.D., Ph.D., Professor of Medicine & Physiology; Co-Director Stem Cell and Regenerative Medicine Center, University of Wisconsin

Human iPSC cells hold great promise for cardiovascular research and therapeutic applications, but the ability of human iPSCs to form functional cardiomyocytes requires careful analysis and optimization. We provide electrophysiological, pharmacological, and biochemical evidence that iPSCs can differentiate into three major types of functional cardiomyocytes which can be used in a variety of applications.

12:00 PM Lunch on Your Own

IPS CELLS FOR DRUG SCREENING

1:00 Chairperson’s Remarks

1:05 Featured Speaker

Stem Cells and Drug Discovery: The Beginning of a New Era?

Lee Rubin, Ph.D., Director, Translational Medicine, Harvard Stem Cell Institute

1:35 CATALYST: The Industrialization of Sponsored by Advanced iPSC Technology for Drug Discovery

Dan Shoemaker, Chief Technology Officer, Fate Therapeutics

CATALYST is designed to accelerate the innovation of induced pluripotent stem cell (iPSC) technology in collaboration with industry to support launching a fully-enabled platform in this new paradigm of drug discovery and development. CATALYST is exploring the creation of iPSC-derived, disease-specific model systems that improve the recapitulation of human physiology and more effectively predict clinical response. CATALYST is committed to developing an iPSC cell technology platform to accelerate candidate identification and lead validation for drug discovery and development for pharmaceutical members. This session will discuss:

- The critical elements of industrialization, including cell sourcing, reprogramming, differentiation and commercial supply
- Approaches for creating non-genetically modified iPSCs and mature phenotypes
- Quantitative methods to analyze cell states for high-quality differentiation and disease modeling
- Uses of iPSC technology in drug discovery

1:50 Sponsored Presentation

Targeting Muscular Dystrophy: How do we Mimic the In Vivo System?

Lorena Grippari, Ph.D., Research Scientist, DV Biologics

Muscular dystrophy (MD) is a well characterized neuromuscular disorder. Here we show that using cells isolated from different tissues of MD patients and their pedigrees is an effective tool for understanding how to treat the disease. Our MD pedigree system is the first commercially available tool allowing the study of this disease and the production of iPSCs.

2:05 iPSC-enabled Drug Discovery: A Paradigm Shift to Increase POS in the Clinic

Berta Strulovici, Chief Technology Officer, iPierian

Until recently, human disease specific pluripotent stem cells could be made only by tedious genetic modification of existing hES cells or by generating such cells from embryos with diagnosed monogenic diseases. Recent advances using induced pluripotent stem cells (iPSCs) have enabled the production of unlimited numbers of cells with a very specific genetic background that can be used as models for drug discovery. Coupled with the ability of these cells to be differentiated to virtually “any type of cell in the body”, the iPSC technology has the ability to revolutionize the way drug discovery is done today. In my presentation, I will describe the use of human iPSC-based assays for drug discovery in our therapeutic areas of focus.

2:20 IPS Cells Panel of Experts

IPS cells have invigorated and united the stem cell research community and strides continue in efficient re-programming. This is evident through funding and companies investing their future through this revolutionary technology. Hear the experts in the IPS Cells field as they present their latest technology followed by an interactive panel discussion.

3:05 Close of Conference
We have carried out an RNA interference-based genome-wide *in vitro* reporter assay screen in *Drosophila* for components of NF-κB pathways. We analyzed 16,025 dsRNA-treatments and identified ten novel NF-κB regulators. Of these, Gprk2 was shown to be evolutionary conserved regulator of NF-κB signalling. siRNA-silencing of human ortholog GRK5 in HeLa cells impaired NF-κB reporter activity. Morpholino-silencing of zebrafish GRK5 homolog in fish embryos caused impaired IL-1β and TNF-α expression after *E. coli* infection. Gprk2/GRK5 was identified as an evolutionarily conserved modulator of NF-κB signaling.

**11:40 Alexander Bishop, Ph.D., Associate Professor, UT San Antonio**

**12:10 PM Generation And Integration of HT-RNAi Screening Data**

Pedro Aza-Blanc, Ph.D., Director, Functional Genomics Resources, The Burnham Institute for Medical Research

**12:40 Luncheon Presentation I**

Using siRNA to Investigate Non-Coding RNA (ncRNA) Function in Control of Mitosis and Apoptosis in Cells

Susan Magdaleno, Ph.D., Senior Manager, Scientist, RNAi Technologies Research & Development, Applied Biosystems

Long non-coding RNAs (ncRNA) are critical to biology and disease. Life Technologies has now developed a suite of integrated tools and workflows to discover, validate, and knock-down ncRNA which will accelerate understanding of the function of ncRNA in the cell. We will describe the special requirements for using siRNAs to knock down ncRNA and will highlight the application of siRNAs to investigate ncRNA function in regulating mitosis and apoptosis in normal and cancer cells.

**1:10 Luncheon Presentation II (Sponsorship Opportunity Available)**

**1:45 Dessert in the Exhibit Hall**
SCREENING AND VALIDATING DRUG TARGETS USING RNAI SCREENS

8:25 AM Chairperson’s Remarks
Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.

8:30 Cancer Target Identification and Validation by siRNA Library Screening
Xiaoyu Lin, Ph.D., Associate Research Investigator, siRNA Therapeutics, Abbott Laboratories
We have been performing large-scale siRNA library screens to identify novel cancer targets. One of the critical aspects of screen data analysis is to discard false positive hits due to siRNA off-target effect. We will discuss several different approaches to confirm on-target effect of siRNA library hits. Using several targets identified in the library screen as examples, we will update on the progress of how RNAi-based technologies have helped target discovery and validation in the oncology area.

9:00 Hitting Cancer Where it Hurts Most: Large Scale RNAi Screens for Cancer Cell Vulnerability
Roderick L. Beijersbergen, Ph.D., Group Leader, Division of Molecular Carcinogenesis, The Netherlands Cancer Institute
Large scale RNAi screens for cancer cell vulnerability RNA interference based technologies allow for the interrogation of the role and phenotype of all individual genes in the human genome. Using these techniques we aim to identify those genes that upon functional inactivation have a causal effect on tumor cell behavior and survival representing novel drug targets.

9:30 Leveraging RNAi and Chemogenomic Screens for Target Identification and Validation
Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.
Libraries of RNAi reagents are being widely used for screening cellular assays for target identification. We are using RNAi libraries in combination with libraries of small molecule tool compounds. Using both types of libraries adds confidence to hits identified from these screens and provides genetic and chemical tools for hit follow up. Examples will be presented to illustrate this approach in target identification and validation.

10:00 Sponsored Presentation (Sponsorship Opportunity Available)

10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

11:30 Panel: Do’s and Don’ts in RNAi Screening
Panelists:
Christophe J. Echeverri, Ph.D., CEO and CSO, Cenix BioScience GmbH
Christopher Miller, Ph.D., Group Director, Applied Genomics, Bristol-Myers Squibb Co.
Hakim Djaballah, Ph.D., Director, HTS Core Facility, Memorial Sloan Kettering Cancer Center
Paul Kassner, Ph.D., Scientific Director, Lead Discovery, Amgen, Inc.

12:30 PM Luncheon Presentation
Sponsored by
RNAi and KinaseSwitch Technology Platforms
Christine L. Olsson, Ph.D., Taconic
Novel in vivo technology platforms have recently been developed that will enable investigators to gain greater insights into drug and target-related disease mechanisms. TaconicArtemis RNAi and KinaseSwitch mouse models are the newest commercially available additions to these technologies. Inducible/reversible RNAi technology enables gene knockdown in all tissues of the body and can be induced and reversed, providing an optimal surrogate for therapeutic drug action. More recently, KinaseSwitch technology, an invaluable tool during key stages of drug development, allows investigators to identify biological roles of a specific kinase and possible side effects that result from its inhibition.

1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction

2:25 Plenary Keynote Presentation (See Page 26 for Details)

3:05 Refreshment Break in the Exhibit Hall

CLINICAL CHALLENGES WITH RNAI THERAPEUTICS

3:45 Chairperson’s Remarks
Cristina Rondinone, Ph.D., Director Research, Metabolic Diseases, Hoffmann La Roche Inc.

3:50 LNA Antimirs – Pioneering microRNA Therapeutics
Henrik Orum, M.Sc., Ph.D., VP and CSO, Santaris Pharma
Short, single stranded LNA oligonucleotides delivered systemically as naked molecules are able to potently and safely inhibit therapeutically attractive miRNAs in a range of tissues in experimental animals. The presentation will provide an update on the unique features of LNA oligonucleotides in miRNA therapeutics with particular emphasis on the pre-clinical and clinical development of SPC3649, an LNA Antimir targeting miRNA-122.

4:20 Pre-clinical and Clinical Development of Atu027 (siRNA-lipoplex/AtuPLEX) for Oncology
Ansgar Santel, Ph.D., Senior Scientist, Silence Therapeutics plc
Atu027 refers to a liposomally formulated siRNA targeting PKN3 expression in the vascular endothelium. Pre-clinical studies in rodents and non-human primates demonstrated that intravenous administration is well tolerated and gives rise to RNAi-mediated suppression of PKN3 gene expression. Various proof-of-concept experiments on mouse tumor xenografts suggest profound inhibition of tumor progression and particularly of metastasis, which laid the foundation for therapeutic application in oncology. Atu027 is currently tested in a Phase-I clinical trial on subjects with advanced solid cancers. Pre-clinical data emphasizing pharmacological activity in mouse models and an update on the current Phase-I study will be discussed.

4:50 Talk Title to be Announced

5:20 Talk Title to be Announced
Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

5:50 Close of Day

FRIDAY, FEBRUARY 5

NOVEL FORMULATIONS FOR RNAi DELIVERY

8:35 Therapeutic siRNA Delivery: Tackling the 800 Pound Gorilla
Steven F. Dowdy, Ph.D., Investigator, Howard Hughes Medical Institute; Professor, Department of Cellular & Molecular Medicine, University of California, San Diego School of Medicine
To date, siRNA delivery remains the rate-limiting step for RNAi therapies development. We developed a Peptide Transduction Domain (PTD)-DRBD fusion protein siRNA delivery approach. PTD-DRBD delivered siRNAs induced RNAi responses in the entire population of all cell types assayed (primary and tumorigenic) in a non-cytotoxic fashion. PTD-DRBD combinatorial in vivo delivery of EGFR and Akt2 siRNAs induced a synthetic lethal response that significantly increased survival of intracerebral glioblastoma pre-clinical models. These observations demonstrate the ability of PTD-DRBD to efficiently deliver siRNAs in vivo.
9:05 Induction of Therapeutic Gene Silencing in Leukocyte-Implicated Diseases by Targeted and Stabilized Nanoparticles
Dan Peer, Ph.D., Head, Laboratory of Nanomedicine, Department of Cell Research and Immunology and the Center for Nanoscale and Nanotechnology, Tel Aviv University

Leukocytes are among the most difficult cells to transduce with RNAi. We developed a strategy that can target different subsets of leukocytes and selectively silence genes in vivo using targeted, stabilized nanoparticles (tsNPs). These carriers do not induce lymphocyte activation, interferon responses or release liver enzymes and are fully degradable. Three preclinical examples inflammatory bowel disease (IBD), blood cancer and viral infection will be discussed. We will show that tsNPs can be used for in vivo validation of new drug targets, for prevention of viral infection and for inducing therapeutic gene silencing in a preclinical setting.

9:35 Characterization of Immune Responses to tkRNAi Therapeutics
Johannes Fuhrhauf, M.D., Ph.D., VP Research, Cepent Pharmaceuticals, Inc.

Transkingdom RNA interference (tkRNAi) describes a novel method for delivery of therapeutic RNA interference into gastrointestinal tissues using engineered bacteria which produce and deliver mediators of RNAi. Clinical trials are about to begin for the prevention of colon Polyposis, and for the treatment of Inflammatory Bowel Disease (IBD). Here we demonstrate recent results from large screening efforts characterizing the effects of tkRNAi on cytokine profiles and innate immunity.

10:05 Sponsored Presentation (Sponsorship Opportunity Available)
10:20 Coffee Break

11:00 Panel: Do We Understand the Challenges We Face With RNAi Therapeutics?
Panelists:
Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.
Ian MacLachlan, Ph.D., CSO, Tekmira Pharmaceuticals
John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope
Steven Highlander, Ph.D., Partner, Intellectual Property, Fulbright & Jaworski, L.L.P.

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

NOVEL APPROACHES FOR TARGETED DELIVERY

1:00 Chairperson’s Remarks
John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

1:05 In vivo delivery of Dicer substrate RNAs for treatment of HIV infection
John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

The application of RNAi for treatment of HIV infection has many advantages over conventional drugs. The inhibitors can be rapidly changed according to the viruses ability to mutate. This presentation will discuss the use of aptamers and dendrimers to deliver Dicer substrate RNAs in vivo. The results obtained demonstrate that Dicer substrate siRNAs can be delivered in a cell type specific manner with an aptamer, and can be generally delivered with dendrimers to effectively inhibit HIV replication in a humanized mouse model.

1:35 Targeted RNA-based Cancer Therapies
Paloma H. Giangrande, Ph.D., Assistant Professor, Department of Internal Medicine, University of Iowa

A major hurdle for the clinical translation of siRNAs into effective therapies is delivery. We describe an RNA aptamer-based approach for the targeted delivery of siRNAs to prostate cancer (PC) cells. The aptamer-siRNA reagent (chimera) is effective when administered systemically and is suitable for efficient chemical synthesis. When administered systemically to mice bearing PSMA-positive tumors, the RNA chimera triggered tumor regression without affecting normal tissues. This work is the first description of in vivo efficacy following systemic administration of an aptamer-siRNA chimera and thus represents a milestone for this platform technology.
Selectivity and efficacy targeting selectivity of bispecific antibodies that co-target two distinct
Program, Fox Chase Cancer Center

2:20 Bispecific antibodies: an approach to enhance targeting
Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine

Studies examining the roles of affinity on demonstrate that affinity also impacts the targeting of intact antibodies. Studies performed with anti-HER2 human IgGs molecules that demonstrate solid tumors less efficiently with increasing affinity. We will describe anti-HER2 scFv molecules penetrate and localize inChase Cancer Center
Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center

We have found that anti-HER2 scFv molecules penetrate and localize in solid tumors less efficiently with increasing affinity. The ability of intact antibodies and fragments to access tumor cells distant from the tumor blood supply is an important therapeutic consideration for antibody based oncology drugs. Pre-clinical and clinical data will be reviewed regarding the impact of size and exposure time on antibody distribution within tumors.

11:40 Anti-tumor Efficacy Maximization through Blocking Multiple Targets of Angiogenesis
Dana Hu-Lowe, Ph.D., Group Leader, Associate Research Fellow, Cancer Biology, Pfizer, Inc., PGRD-La Jolla

Vascular normalization and adaptivity potentially contribute to resistance to anti-VEGF/VEGFR therapies in the clinic. Other targets, including the Activin receptor Like Kinase 1 (ALK-1), also play a role in promoting tumor angiogenesis. A fully human mAb against ALK-1 was generated. The differential and complimentary outcome of anti-ALK-1 and anti-VEGF will be discussed.

12:10 Tumor Penetration of Therapeutic Antibodies -The Impact of Size and Exposure Time on Distribution
David Blakey, Ph.D., Chief Scientist, Cancer and Infection Research Area, AstraZeneca

The ability of intact antibodies and fragments to access tumor cells distant from the tumor blood supply is an important therapeutic consideration for antibody based oncology drugs. Pre-clinical and clinical data will be reviewed regarding the impact of size and exposure time on antibody distribution within tumors.

3:20 A Systems Biology Approach to Engineering Therapeutic Antibodies: Development of an ErbB2/ErbB3 Bispecific Antibody
Alexandra Huhalov, Ph.D., Principal Scientist, Merrimack Pharmaceuticals

Using quantitative biological datasets of cell signaling we have generated computational models of the ErbB signaling network and identified ErbB3 as a promising target. The application of these models to guide the design of MM-111, a bispecific antibody-based therapeutic targeting the ErbB2/ErbB3 heterodimer, and its antitumor activity will be discussed.

3:50 Large Volume Subcutaneous Delivery: Challenges and Opportunities
Robin Hwang, Ph.D., Executive Director, Halozyme

There are many monoclonal antibodies (mAbs) in development for cancer therapeutics. Generally, mAbs require a higher dosage than the typical protein therapeutics. It has been shown clinically that the “standard” subcutaneous injection can go up to 1.5 mL, beyond which skin distortion and pain can occur. As a result, most biotech companies spend much effort in concentrating Mabs to ≈100 mg/mL and then trying to stabilize these formulations to avoid aggregates and particulates. Halozyme’s Enhance Technology permits the large volume subcutaneous (SC) dosing, with corresponding lower protein concentration which was not previously feasible. Bypassing high-concentration formulation challenges has the potential to accelerate the timeline to bring a product to the clinic, enables an IV-SC switch, in some cases improves bioavailability, and improve patient convenience and compliance. In this talk, large volume SC delivery and device options will be presented.

2:50 Selective Penetration and Targeting of Tumors
Tapas K. Das Gupta, M.D., Ph.D., D.Sc., Professor and Head, Surgical Oncology, University of Illinois Chicago; Co-founder, CDG Therapeutics, Inc.

CDG Therapeutics has developed a cell penetrating peptide (28aa) from azurin, a redox protein secreted by Pseudomonas aeruginosa. p28 preferentially enters cancer cells, localizes in the nucleus and stabilizes p53 inducing cell cycle arrest and apoptosis in a series of solid tumors. p28 is stable, nontoxic and currently in a Phase I clinical trial.

2:15 Chairperson’s Remarks
Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine Program, Fox Chase Cancer Center

Work will be presented on our efforts to develop and optimize the targeting selectivity of bispecific antibodies that co-target two distinct tumor associated antigens. We hypothesize that the targeting selectivity afforded by these molecules can potentially be leveraged for the development of new immunodrug conjugates.

8:30 Tumor Targeting Theory-Kinetic & Diffusive Processes that Determine Antibody Macro & Microdistribution
K. Dane Wittrup, Ph.D., C.P. Dubbs Professor, Chemical Engineering & Biological Engineering, Associate Director, Koch Institute for Integrative Cancer Research, Massachusetts Institute of Technology

A diverse array of tumor targeting agents ranging in size from peptides to nanoparticles is currently under development for applications in cancer imaging and therapy. However, it remains largely unclear how size differences among these molecules influence their targeting properties. Here
we develop a simple, mechanistic model that can be used to understand and predict the complex interplay between molecular size, affinity, and tumor uptake.

9:00 Nanoparticle Agents for Tumor Targeting and Penetration
Shuming Nie, Ph.D., Wallace H. Coulter Distinguished Faculty Chair in Biomedical Engineering, Director of Emory-Georgia Tech Cancer Nanotechnology Center, Professor of BME, Chemistry, Materials Science and Engineering, and Hematology and Oncology, Emory University and Georgia Institute of Technology

Nanoparticles have functional and structural properties not available from discrete molecules or bulk materials. When conjugated with monoclonal antibodies, peptides or small molecules, nanoparticles can be used to target malignant tumors with high specificity and affinity. We developed a new class of biocompatible and nontoxic nanoparticles for in vivo tumor targeting and detection based on self-assembled nanostructures and pegylated colloidal gold.

9:30 Delivery of Antibodies – Market Analysis & Overview
Tugrul Kararli, Ph.D., President & Founder, Pharmacircle

10:00 Sponsored Presentation (Sponsorship Opportunity Available)
10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

DELIVERY OF ANTIBODIES

11:30 Engineered Antibodies for Molecular Imaging of Cancer
Anna M. Wu, Ph.D., Professor, David Geffen School of Medicine at UCLA
Cancer-targeting antibodies have been optimized for in vivo imaging by conversion into fragments such as diabodies, minibodies, and scFv-Fc. Recombinant fragments recognizing a variety of cell-surface markers have been labeled with positron-emitting radionuclides (I-124, Cu-64, and F-18) for positron-emission tomography (PET) detection of tumors in living organisms, including target expression, target coverage, and response to therapy.

12:00 Advanced Polymer Conjugate Technology for Optimization of Cancer Therapeutics
Christine Loehrlein, Ph.D., A.D., New Products and Technology Strategy Research, Nektar Therapeutics
Conjugation of a therapeutic agent to polyethylene glycol and other polymers is a general strategy that can be used to optimize pharmacological parameters of that drug, with the ability to affect both its efficacy and side effect profile. Nektar’s Advanced Polymer Conjugate Technology platform can be used to enable a wide range of molecules, including proteins, peptides, small molecule oral and parenteral drugs, and antibody fragments. Nektar is currently using this approach to develop advanced oncolytic constructs with sustained exposure to tumor cells, and exploring opportunities to extend this technology to other cancer therapeutics.

12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own
1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction
2:25 Plenary Kenyote Presentation (See Page 26 for Details)
3:05 Refreshment Break in the Exhibit Hall

NOVEL MODES OF ACTION FOR CANCER BIOTHERAPEUTICS
BI- AND TRI-SPECIFIC ANTIBODIES

3:45 Chairperson’s Remarks
Stefan Dübel, Ph.D., Director, Biotechnology, Technische Universität Braunschweig, Germany

3:50 Mode of BiTE Antibody Action in Cancer Therapy
Patrick Baeuerle, Ph.D., CSO and Senior Vice President, Research & Development, Micromet AG
BiTE (bispecific T cell engager) antibody blinatumumab targets CD19 on B cell malignancies and has provided clinical proof of concept in phases 1 and 2. We will discuss the mode of BiTE antibody action in inducing highly efficient cancer cells lysis, and provide background on how BiTE antibodies are produced, are administered in the clinic, and have been pre-clinically developed.

4:20 Catumaxomab (Removab), the First EC-approved Trifunctional Bispecific Antibody: The Road from Pre-clinical Development to Approval and Beyond
Diane Seimetz, Ph.D., M.D.R.A., CSO and Executive Vice President, Fresenius Biotech GmbH
Catumaxomab, a targeted therapy for intraperitoneal treatment of malignant ascites, targets the epithelial cell-adhesion molecule (EpCAM) and CD3 evoking T-cell cytotoxicity on EpCAM-expressing tumor cells. The Fc-region of catumaxomab provides a third functional binding site, which binds and activates Fcy-receptor-positive accessory cells. The development rationale, pre-clinical and clinical data, approval process and preparations for further clinical development will be presented.

4:50 Bispecific EGFR-IGF1R Program
Eric Furfine, Ph.D., Senior Vice President, Research and Pre-clinical Development, Adnexus Therapeutics, a Bristol-Myers Squibb R&D Company
Adnectins offer several potential advantages compared to traditional targeted biologics, including speed of discovery, efficient manufacturing, and the ability to create multi-functional targeted products. We are currently advancing products combining two Adnectins to enable modulation of two distinct targets. We will present methods to engineer and optimize multi-specific Adnectins, and pre-clinical data on a bispecific Adnectin to EGFR and IGF1R.

5:20 Multi-specific Antibody by Design
Changshou Gao, Ph.D., Principal Scientist, Antibody Discovery and Protein Engineering, MedImmune LLC
We’ll discuss efforts to engineer and optimize multispecific antibody formats to address the challenges pertaining to bispecific and multispecific molecules, and provide data of our multispecific constructs with excellent expression level, great biophysical stability, good in vitro and in vivo activities, and potential manufacturing feasibility. Our multispecific constructs retain similar in vivo half-life and effector functions to their parental antibodies.

5:50 Close of Day
8:00 Chairperson’s Opening Remarks
Hans-Peter Gerber, Ph.D., Senior Director, Tumor Therapies, Wyeth Oncology Discovery

8:05 Pre-clinical and Clinical Development of Calicheamicin Derivatives Conjugated to Monoclonal Antibodies
Hans-Peter Gerber, Ph.D., Senior Director, Tumor Therapies, Wyeth Oncology Discovery

8:35 A Novel Minor Groove Binding Alkylation Agent for Antibody Targeted Chemotherapy of CD70 Expressing Cancers
Nils Lonberg, Ph.D., Senior Vice President and Scientific Director, Medarex, Inc.

An antibody drug conjugate comprising a CD70 targeting monoclonal antibody and a novel alkylation agent is now in Phase I clinical development for kidney cancer and lymphoma. The mechanism of action of this novel therapeutic, activated through a multistep mechanism including esterase mediated removal of a blocking group and protease mediated release of the cytotoxic drug, will be discussed.

9:05 Clinical and Preclinical Evaluation of Calicheamicin-Linked Antibody Drug Conjugates
Hans-Peter Gerber, Ph.D., Senior Director, Tumor Therapies, Wyeth Oncology Discovery

9:35 Antibody-Maytansinoid Conjugates: Demonstrating Benefit in the Treatment of Solid and Liquid Tumors
John M. Lambert, Ph.D., Executive Vice President and CSO, ImmunoGen, Inc.

Several highly potent cell-killing agents such as derivatives of the anti-mitotic microtubule agent, maytansine, are currently being utilized in ADCs to achieve effective, well tolerated anticancer drugs. Several AMCs show encouraging efficacy in clinical trials, including T-DM1, currently being developed by Genentech using ImmunoGen’s maytansinoid technology. New payloads for ADCs are realizing the promise of antibody-mediated delivery in cancer.

10:05 Broad Application of Scaffold Antibodies for Targeted Tumor Therapy
Gary Woodnutt, Ph.D., Vice President, Biology, CovX Research, A Pfizer organization

The progression of novel cancer therapeutics that have the potential of truly impacting disease requires the identification of targets that affect tumor growth combined with a modality capable of rapid exploitation of those targets either as monotherapy or in combination. We will describe how the use of bioconjugation to a proprietary scaffold antibody allows us to develop these therapeutics rapidly and effectively.

10:20 Coffee Break

10:30 Pre-clinical and Clinical Evaluation of Calicheamicin-Linked Antibody Drug Conjugates
Hans-Peter Gerber, Ph.D., Senior Director, Tumor Therapies, Wyeth Oncology Discovery

11:00 Bi-specific, High Affinity T Cell Receptor Fusions as Anti-Cancer Therapeutics
Rebecca Ashfield, D.Phil., Senior Research Project Manager, Immunocore Ltd

Bi-specific TCRs, consisting of high affinity T cell receptors fused to an anti-CD3 scFv, are being developed for the treatment of several tumor types. The presentation will cover engineering of these reagents, demonstration of efficacy including animal models, and a discussion of the planned first-in-man clinical trial including toxicity testing, a challenge since the molecules are entirely human specific.

11:30 Cancers

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

EFFECTOR-ENHANCED BIOTherAPEUTICS

1:00 Chairperson’s Remarks
John F. McDonald, Professor and Director, Integrated Cancer Research Center, School of Biology, Georgia Institute of Technology

1:05 Human RNase Fusion Proteins for Tumor Therapy
Stefan Dübel, Ph.D., Director, Biotechnology, Technische Universität Braunschweig, Germany

RNases are non-toxic while in circulation but highly effective in cell killing after targeted internalization. An entirely human immunoenzyme against CD30+ lymphomas was constructed from a human scFv-Fc antibody fragment and a human RNase. It did not affect the human embryonal kidney used for its production but strongly inhibited proliferation of CD30+ lymphoma cells.

1:30 Glycoengineering for the Enhancement of Antibody Activity
Dennis Benjamin, Ph.D., Senior Director, Antibody Technologies, Seattle Genetics, Inc.

2:05 Antibody Fc Engineering to Enhance Cytotoxicity, Pharmacokinetics, and Pharmacodynamics
John R. Desjarlais, Ph.D., VP, Research, Xencor, Inc.

We have engineered the antibody Fc domain to enhance its affinity for Fc receptors, leading to a set of variants that confer high ADCC activity onto antibodies targeting a wide range of tumor targets. These variants also enhance anti-tumor activity in mouse models and cynomolgus monkeys. A phase I trial is underway to determine their effects in humans.

2:35 Arzerra™ (ofatumumab), a Novel Human Therapeutic CD20-Antibody: Mechanisms of Action and Efficacy in B-CLL
Frank Beurskens Ph.D., Senior Scientist, Strategic Research, Gennab, B.V.

We developed a unique human monoclonal CD20 antibody, ofatumumab, that targets a distinct epitope encompassing both the small and large extracellular loops of the CD20 molecule and displays an exceptional efficacy in inducing complement dependent cell lysis (CDC). Novel insights into the mechanism of tumor cell killing by ofatumumab and its efficacy in clinical trials in B-CLL will be discussed.

3:05 Close of Conference

ALUMNI DISCOUNT
Cambridge Healthtech Institute (CHI) appreciates your past participation at the Molecular Medicine Tri-Conference. Through loyalty like yours, CHI has been able to build this event into a must-attend for senior level decision-makers. As a result of the great loyalty you have shown, we are pleased to extend to you the exclusive opportunity to save an additional 20% off the registration rate. Just check off the box marked Alumni Discount on the registration form to receive the discount! Please note: Our records must indicate you were an attendee of the Tri-Conference event in the past in order to qualify.
2:00 AM Registration and Morning Coffee
8:00 Plenary Keynote Session (See Page 26 for Details)
9:40 Grand Opening Refreshment Break in the Exhibit Hall

WEDNESDAY, FEBRUARY 3

11:00 Introduction and Welcome
Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center

11:10 Tumor Penetration of Therapeutic Antibodies - The Impact of Size and Exposure Time on Distribution
David Blakey, Ph.D., Chief Scientist, Cancer and Infection Research Area, AstraZeneca

The ability of intact antibodies and fragments to access tumor cells distant from the tumor blood supply is an important therapeutic consideration for antibody-based oncology drugs. Preclinical and clinical data will be reviewed regarding the impact of size and exposure time on antibody distribution within tumors.

11:40 Anti-tumor Efficacy Maximization through Blocking Multiple Targets of Angiogenesis
Dana Hu-Lowe, Ph.D., Group Leader, Associate Research Fellow, Cancer Biology, Pfizer, Inc., PGRD-La Jolla

Vascular normalization and adaptivity potentially contribute to resistance to anti-VEGF/VEGFR therapies in the clinic. Other targets, including the Activin receptor Like Kinase 1 (ALK-1), also play a role in promoting tumor angiogenesis. A fully human mAb against ALK-1 was generated. The differential and complimentary outcome of anti-ALK-1 and anti-VEGF will be discussed.

12:10 PM Optimizing Targeting of Anti-tumor Antibodies
Gregory P. Adams, Ph.D., Co-Leader, Molecular and Translational Medicine Program, Fox Chase Cancer Center

We have found that anti-HER2 scFv molecules penetrate and localize in a solid tumors less efficiently with increasing affinity. We will describe studies performed with anti-HER2 human IgGs molecules that demonstrate that affinity also impacts the targeting of intact antibodies. Studies examining the roles of affinity on in vitro ADCC and internalization into tumor cells will also be discussed.

12:40 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

THURSDAY, FEBRUARY 4

2:20 Bispecific Antibodies: An Approach to Enhance Targeting Selectivity and Efficacy
Matthew K. Robinson, Ph.D., Assistant Professor, Molecular and Translational Medicine Program, Fox Chase Cancer Center

Work will be presented on our efforts to develop and optimize the targeting selectivity of bispecific antibodies that co-target two distinct tumor associated antigens. We hypothesize that the targeting selectivity afforded by these molecules can potentially be leveraged for the development of new immunodrug conjugates.

2:50 Selective Penetration and Targeting of Tumors
Tapas K. Das Gupta, M.D., Ph.D., D.Sc., Professor and Head, Surgical Oncology, University of Illinois Chicago; Co-founder, CDG Therapeutics, Inc.

CDG Therapeutics has developed a cell penetrating peptide (28aa) from azurin, a redox protein secreted by Pseudomonas aeruginosa. p28 preferentially enters cancer cells, localizes in the nucleus and stabilizes p53 inducing cell cycle arrest and apoptosis in a series of solid tumors. p28 is stable, nontoxic and currently in a Phase I clinical trial.

3:20 A Systems Biology Approach to Engineering Therapeutic Antibodies: Development of an ErbB2/ErbB3 Bispecific Antibody
Alexandra Huhalov, Ph.D., Principal Scientist, Merrimack Pharmaceuticals

Using quantitative biological datasets of cell signaling we have generated computational models of the ErbB signaling network and identified ErbB3 as a promising target. The application of these models to guide the design of MM-111, a bispecific antibody-based therapeutic targeting the ErbB2/ErbB3 heterodimer, and its antitumor activity will be discussed.

3:50 Sponsored Presentation (Sponsorship Opportunity Available)
4:20 Reception in the Exhibit Hall (Sponsorship Available)
5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall
6:20 Close of Day

ENGINEERING FOR DELIVERY

8:25 AM Chairperson’s Remarks
Tugral Karafi, Ph.D., President & Founder, Pharmacircle

8:30 Tumor Targeting Theory-Kinetic & Diffusive Processes that Determine Antibody Macro & Microdistribution
K. Dane Wittrup, Ph.D., C.P. Dubbs Professor; Chemical Engineering & Biological Engineering, Associate Director, Koch Institute for Integrative Cancer Research, Massachusetts Institute of Technology

A diverse array of tumor targeting agents ranging in size from peptides to nanoparticles is currently under development for applications in cancer imaging and therapy. However, it remains largely unclear how size differences among these molecules influence their targeting properties. Here we develop a simple, mechanistic model that can be used to understand and predict the complex interplay between molecular size, affinity, and tumor uptake.

9:00 Nanoparticle Agents for Tumor Targeting and Penetration
Nanoparticles have functional and structural properties not available from discrete molecules or bulk materials. When conjugated with monoclonal antibodies, peptides or small molecules, nanoparticles can be used to target malignant tumors with high specificity and affinity. We developed a new class of biocompatible and nontoxic nanoparticles for in vivo tumor targeting and detection based on self-assembled nanostructures and pegylated colloidal gold.

9:30 Delivery of Antibodies – Market Analysis & Overview
Tugral Kararli, Ph.D., President & Founder, Pharmacircle

10:00 Sponsored Presentation (Sponsorship Opportunity Available)
10:30 Poster Competition Refreshment Break & Raffles in the Exhibit Hall

DELCIVERY OF ANTIBODIES

11:30 Engineered Antibodies for Molecular Imaging of Cancer
Anna M. Wu, Ph.D., Professor, David Geffen School of Medicine at UCLA
Cancer-targeting antibodies have been optimized for in vivo imaging by conversion into fragments such as diabodies, minibodies, and scFv-Fc. Recombinant fragments recognizing a variety of cell-surface markers have been labeled with positron-emitting radionuclides (I-124, Cu-64, F-18) for positron-emission tomography (PET) detection of tumors in preclinical models. ImmunoPET represents a broad platform for conducting "immunohistochemistry in vivo" to address biological questions in living organisms, including target expression, target coverage, and response to therapy.

12:00 Advanced Polymer Conjugate Technology for Optimization of Cancer Therapeutics
Christine Lochstein, Ph.D., A.D., New Products and Technology Strategy Research, Nektar Therapeutics
Conjugation of a therapeutic agent to polyethylene glycol and other polymers is a general strategy that can be used to optimize pharmacological parameters of that drug, with the ability to affect both its efficacy and side effect profile. Nektar's Advanced Polymer Conjugate Technology platform can be used to enable a wide range of molecules, including proteins, peptides, small molecule oral and parenteral drugs, and antibody fragments. Nektar is currently using this approach to develop advanced oncolytics with sustained exposure to tumor cells, and exploring opportunities to extend this technology to other cancer therapeutics.

12:30 Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

1:45 Ice Cream Refreshment Break in the Exhibit Hall

PLENARY KEYNOTE SESSION

2:15 Plenary Keynote Introduction
2:25 Plenary Keynote Presentation (See Page 26 for Details)
3:05 Refreshment Break in the Exhibit Hall

CLINICAL CHALLENGES WITH RNAI THERAPEUTICS

3:45 Chairperson's Remarks
Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

3:50 LNA Antimir – Pioneering microRNA Therapeutics
Henrik Orum, M.Sc., Ph.D., VP and CSO, Santaris Pharma
Short, single stranded LNA oligonucleotides delivered systemically as naked molecules are able to potently and safely inhibit therapeutically attractive miRNAs in a range of tissues in experimental animals. The presentation will provide an update on the unique features of LNA oligonucleotides in miRNA therapeutics with particular emphasis on the pre-clinical and clinical development of SPC3649, an LNA AntimiR targeting miRNA-122.

4:20 Pre-clinical and Clinical Development of Atu027 (siRNA-lipoplex/AtuPLEX) for Oncology
Klaus Giese, Ph.D., CSO, Silence Therapeutics plc
Atu027 refers to a liposomally formulated siRNA targeting PKN3 expression in the vascular endothelium. Pre-clinical studies in rodents and non-human primates demonstrated that intravenous administration is well tolerated and gives rise to RNAi-mediated suppression of PKN3 gene expression. Various proof-of-concept experiments on mouse tumor xenografts suggest profound inhibition of tumor progression and particularly of metastasis, which laid the foundation for therapeutic application in oncology. Atu027 is currently tested in a Phase-I clinical trial on subjects with advanced solid cancers. Pre-clinical data emphasizing pharmacological activity in mouse models and an update on the current Phase-I study will be discussed.

4:50 Talk Title to be Announced
Ian MacLachlan, Ph.D., CSO, Tekmira Pharmaceuticals

5:20 Talk Title to be Announced
Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.

5:50 Close of Day

FRIDAY, FEBRUARY 5

NOVEL FORMULATIONS FOR RNAI DELIVERY

8:30 AM Chairperson's Opening Remarks
C. Satishchandran, Ph.D., Chief Technology Officer, Research Technology Center, Pfizer, Inc.

8:35 Therapeutic siRNA Delivery: Tackling the 800 Pound Gorilla
Steven F. Dowdy, Ph.D., Investigator, Howard Hughes Medical Institute; Professor, Department of Cellular & Molecular Medicine, University of California, San Diego School of Medicine
To date, siRNA delivery remains the rate-limiting step for RNAi therapeutics development. We developed a Peptide Transduction Domain-siRNA Binding Domain (PTD-DRBD) fusion protein siRNA delivery approach. PTD-DRBD delivered siRNAs induced RNAi responses in the entire population of all cell types assayed (primary and tumorigenic) in a non-cytotoxic fashion. PTD-DRBD combinatorial delivery of EGFR and Akt2 siRNAs induced a synthetic lethal response that significantly increased survival of intracerebral glioblastoma pre-clinical models. These observations demonstrate the ability of PTD-DRBD to efficiently deliver siRNAs.

9:05 Induction of Therapeutic Gene Silencing in Leukocyte-Implicated Diseases by Targeted and Stabilized Nanoparticles
Dan Peer, Ph.D., Head, Laboratory of Nanomedicine, Department of Cell Research and Immunology and the Center for Nanoscience and Nanotechnology, Tel Aviv University
Leukocytes are among the most difficult cells to transduce with RNAi. We developed a strategy that can target different subsets of leukocytes and selectively silence genes in vivo using targeted, stabilized nanoparticles (tsNPs). These carriers do not induce lymphocyte activation, interferon responses or release liver enzymes and are fully degradable. Three preclinical examples inflammatory bowel disease (IBD), blood cancer and viral infection will be discussed. We will show that tsNPs can be used for in vivo validation of new drug targets, for prevention of viral infection and for inducing therapeutic gene silencing in a preclinical setting.
9:35 Characterization of Immune Responses to tkRNAi Therapeutics
Johannes Fruehauf, M.D., Ph.D., VP, Research, Cequent Pharmaceuticals, Inc.
Transkingdom RNA interference (tkRNAi) describes a novel method for delivery of therapeutic RNA interference into gastrointestinal tissues using engineered bacteria which produce and deliver mediators of RNAi. Clinical trials are about to begin for the prevention of colon Polyposis, and for the treatment of Inflammatory Bowel Disease (IBD). Here we demonstrate recent results from large screening efforts characterizing the effects of tkRNAi on cytokine profiles and innate immunity.

10:05 Sponsored Presentation (Sponsorship Opportunity Available)

10:20 Coffee Break

11:00 Panel: Do We Understand the Challenges We Face With RNAi Therapeutics?
Panelists:
- Bob D. Brown, Ph.D., Senior Vice President, Research, Dicerna Pharmaceuticals Corp.
- Dmitry Samorsky, Ph.D., VP, Technology Development, RXi Pharmaceuticals
- Ian MacLachlan, Ph.D., CSO, Tekmira Pharmaceuticals
- John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope
- Steven Highlander, Ph.D., Partner, Intellectual Property, Fulbright & Jaworski, L.L.P.

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

NOVEL APPROACHES FOR TARGETED DELIVERY

1:00 Chairperson’s Remarks
John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope

1:05 In vivo delivery of Dicer substrate RNAs for treatment of HIV infection
John Rossi, Ph.D., Professor and Chair, Molecular Biology, Beckman Research Institute of the City of Hope
The application of RNAi for treatment of HIV infection has many advantages over conventional drugs. The inhibitors can be rapidly changed according to the viruses ability to mutate. This presentation will discuss the use of aptamers and dendrimers to deliver Dicer substrate RNAs in vivo. The results obtained demonstrate that Dicer substrate siRNAs can be delivered in a cell type specific manner with an aptamer, and can be generally delivered with dendrimers to effectively inhibit HIV replication in a humanized mouse model.

1:35 Targeted RNA-based Cancer Therapies
Paloma H. Giangrande, Ph.D., Assistant Professor, Department of Internal Medicine, University of Iowa
A major hurdle for the clinical translation of siRNAs into effective therapies is delivery. We describe an RNA aptamer-based approach for the targeted delivery of siRNAs to prostate cancer (PC) cells. The aptamer-siRNA reagent (chimera) is effective when administered systemically and is suitable for efficient chemical synthesis. When administered systemically to mice bearing PSMA-positive tumors, the RNA chimera triggered tumor regression without affecting normal tissues. This work is the first description of in vivo efficacy following systemic administration of an aptamer-siRNA chimera and thus represents a milestone for this platform technology.

2:05 Development of Novel Therapeutic RNAi Compounds and Effective in vivo Delivery Approaches
Dmitry Samorsky, Ph.D., VP, Technology Development, RXi Pharmaceuticals
RNA interference (RNAi) offers a novel approach to the drug development process, because RNAi compounds can potentially be designed to target any one of the genes in the human genome. Other potential advantages of RNAi therapeutics include, rapid development of lead compounds, high selectivity for the target gene, high potency (low dose) and low toxicity due to natural mechanism of action. We will introduce unique single-oligo (rxRNA™) and short-duplex (rxRNA™) RNAi compounds, as well as novel in vivo delivery approaches, including self-delivering rxRNA molecules (sd-rxRNA) for local and systemic delivery, and targeted delivery to phagocytic immune cells using.

2:35 Talk Title to be Announced
C. Satishchandran, Ph.D., Chief Technology Officer, Research Technology Center, Pfizer, Inc.

3:05 Close of Conference
TRENDS IN TRANSLATIONAL MEDICINE

11:00 Chairperson’s Remarks
Christina M. Coughlin, M.D., Ph.D.; Medical Director, Oncology; Clinical Research and Development; Pfizer Oncology

11:10 Translational Approach to Studying Stroke
Giora Z. Feuerstein, M.D., Assistant VP, Discovery Translational Medicine, Wyeth Research Labs

To improve success of clinical trials and speed drug development, departments of Translational Medicine in pharma have formed to figure out which molecular, biochemical and physiological biomarkers can best substitute for the absence of clinical outcome studies. My presentation will illustrate how we’ve applied a translational approach to develop better therapies for stroke. Specifically we’ve focused on reducing attrition rate of compounds/biologicals by optimizing 1. Target Validation; 2. Compound -Target interaction; 3. innovative Pharmacokinetic-Pharmacodynamic and proof of Mechanism of Action (MoA); 4. disease biomarkers; 5. Patient selections for clinical trials based on evidence for likelihood to respond to treatment.

11:40 Contribution of Translational Approaches to Recent Advances in Immuno-therapeutics, Immuno-rejection and Beyond
Francesco Marincova, M.D., Chief, Infectious Disease and Immunogenetics Section, NIH; Editor in Chief, Journal of Translational Medicine

The complexity underlying a pathological process does not necessarily require complex solutions. The biology determining allograft or cancer rejection, autoimmunity or tissue damage during pathogen infections is complex; however, common patterns are emerging that lead to a common final outcome: tissue destruction with resolution of the pathogenic process (cancer, infection) or tissue damage and organ failure (allograft rejection, autoimmunity). Human observations based on transcriptional profiling converge into an “immunological constant of rejection” that signals such occurrences. This constant includes the coordinate activation of interferon stimulated genes (ISGs) and immune effector functions (IEFs). Understanding this final effector pathway may suggest novel strategies for the induction or inhibition of tissue-specific destruction with therapeutic intent in cancer and other immune pathologies. This presentation will discuss how vaccines may play a role in tissue-specific destruction and use this as a model to demonstrate how to understand the dynamics of therapeutics by studying target tissues in real time.

12:10 PM Panel: Practical Translational Medicine
Moderator: Vivek Kadambi, Ph.D., Senior Director, Millenium Pharmaceuticals

• When to use biomarkers for go/no-go decisions on proceeding with development of a clinical compound
• How has translational medicine changed over the past 5 years?
• Fostering partnerships between industry, academics and government granting institutions
• How are pharmaco-dynamic and predictive markers being used right now in clinical development?
Panelists: Giora Z. Feuerstein, M.D., Assistant Vice President and Head, Discovery

12:40 Luncheon Presentation I
ZDSD Rat: A Model for Diabetes, Metabolic Syndrome, and Obesity without Leptin or Leptin Receptor Mutations

• The ZDSD rat is a model for obesity, insulin resistance, metabolic syndrome and diabetes with a normal leptin axis.
• The disease conditions expressed in the ZDSD more closely resemble the human situation when compared to other animal models.
• The obesity and diabetes in the ZDSD rat can be modulated with diet.
• The obesity and diabetes in the ZDSD rat can be treated with standard pharmaceuticals.
• The ZDSD rat expresses the bone, renal and other complications seen in diabetes.

1:10 Luncheon Presentation II (Sponsorship Opportunity Available)
WORKING BACKWARDS IN CANCER: FROM THE CLINIC TO DISCOVERY

2:15 Chairperson’s Remarks
Michael Liebman, Ph.D., Managing Director, Strategic Medicine, Inc.

2:20 Using Drug-Induced Feedback Loops to Identify Indications and Combination Partners
Donald Bergstrom, Ph.D., Director, Experimental Medicine Oncology, Merck

James W. Walters, Ph.D., Associate Director, Molecular Profiling Oncology, Merck

Treatment with molecular targeted agents can result in compensatory feedback regulation as cells respond to inhibition of signaling pathways. We will present clinical evidence that treatment with a small molecule inhibitor of gamma secretase results in pathway modulation and compensatory feedback, and describe pre-clinical experiments designed to leverage this concept for drug response prediction.

3:00 Moving Research Closer to the Bedside, in vitro and in vivo Analyses with Primary Tumors
Fred Poordad, M.D., Chief of Hepatology, Liver Disease and Transplant Center, Cedars-Sinai Medical Center, Xin Wei Wang, Ph.D., Senior Investigator Head, Liver Carcinogenesis Section, Laboratory of Human Carcinogenesis, National Cancer Institute, NIH, and Michael R. Briggs, Ph.D., Senior Director, Biology, Vertex Pharmaceuticals, Inc.

The incidence of Primary Liver Cancer is increasing in the west and constitutes a tremendous burden on world health as the third leading cause of cancer deaths worldwide. The 5 year survival rate is a dismal 11 %, due in large part to late diagnosis and limited treatment options. The etiology of this devastating disease as well as current and proposed new therapies will be discussed. Steps to better diagnosis and stratify patients for targeted therapy will be considered as a new and exciting phase of cancer research. Finally, a move toward more relevant research will be presented as an hypothesis that will be tested in the coming years as more new and current therapies are compared and contrasted to current best practice.
In drug discovery and development what really counts is the clinical outcome, the Benefit/Risk of the drug within the context of its pathway or mechanism of action (MoA). Biovista screens the MoA of any drug or target against the MoA of 8,000 indications and 12,000 adverse events (AEs). This is simultaneous, unbiased indication discovery and AE profiling, and it is unique. It helps to bridge the gap from molecular pathway to clinical outcome in a single step. Case studies in cancer and other diseases will be described.

**4:35 Reception in the Exhibit Hall (Sponsorship Available)**

**5:20 BREAK-OUT DISCUSSIONS in the Exhibit Hall**

1. **Novel Imaging Biomarkers in Drug Development**
   - **Moderator:** Jingsong Wang, M.D., Director of Immunology, Discovery Medicine & Clinical Pharmacology, Bristol-Myers Squibb, Co.
   - **Co-Moderators:**
     - Christina M. Coughlin, M.D., Ph.D.; Medical Director, Oncology; Clinical Research and Development; Pfizer Oncology
     - Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson
   - The distinct advantage and unique challenges in applying imaging biomarkers in drug development
   - The most promising novel imaging biomarkers for drug development, and which therapeutic areas will benefit the most from using imaging biomarker
   - The role of pharmaceutical company, CRO, academia and regulatory agency in the discovery, development and qualification of imaging biomarkers

2. **Biomarkers in Translational Medicine**
   - **Moderator:** Yali Fu, Ph.D., Program Director, Grants and Contracts Operations Branch, Developmental Therapeutics Program, Division of Cancer Treatment and Diagnosis, National Cancer Institute
   - **Co-Moderators:**
     - Under NIH Enhanced Peer Review
     - NCI’s new initiatives on SBIR funding
     - Phase II Bridge awards to help biotechs further develop their technologies

3. **New Funding Opportunities for Biotechs**
   - **Moderator:** Troy A. Gobbett, MS, Director, Sales & Marketing for PreClinOmics, Inc.
   - **Co-Moderators:**
     - Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson
     - Charles River
     - Johnson
     - The role of pharmaceutical company, CRO, academia and regulatory agency in the discovery, development and qualification of imaging biomarkers

4. **New Animal Models in Translational Medicine**
   - **Moderator:** Matthew Silva, Ph.D., Head, Imaging Sciences, Millennium, The Takeda Oncology Company
   - **Co-Moderators:**
     - Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson
     - Development
     - The role of pharmaceutical company, CRO, academia and regulatory agency in the discovery, development and qualification of imaging biomarkers

5. **Standardized Solutions for Non-Invasive Imaging of Cell Trafficking**
   - **Moderator:** Jingsong Wang, M.D., Director of Immunology, Discovery Medicine & Clinical Pharmacology, Bristol-Myers Squibb, Co.
   - **Co-Moderators:**
     - Hans Winkler, Ph.D., Senior Director, Global Head Oncology Biomarkers, Johnson & Johnson
     - The role of pharmaceutical company, CRO, academia and regulatory agency in the discovery, development and qualification of imaging biomarkers

**6:20 Close of Day**
**3:50 An Animal Model of Parkinson’s Disease Psychosis: Assessing Potential Therapeutic Efficacy of 5-HT2A Inverse Agonists**  
Krista McFarland, Ph.D., Team Leader, In Vivo Pharmacology, ACADIA Pharmaceuticals, Inc.

Available antipsychotic drugs do not provide an ideal treatment for psychosis in Parkinson’s disease (PDP) because their blockade of dopamine receptors counteracts the dopamine replacement therapy used to alleviate the motor symptoms of PD. Development of alternate pharmacotherapies is limited by the lack of an animal model. Recent efforts to develop such a model and assess the potential therapeutic efficacy of 5-HT2A inverse agonists for the treatment of PDP will be discussed.

**4:20 Engineered Human-In-Mouse Tumors for Population Based in vivo Biomarker Discovery**  
Min Wu, Ph.D., Principal Scientist, Translational Research, AVEO Pharmaceuticals, Inc.

I will present a NEW population-based tumor model system using Human-in-Mouse tissue transgenic human tumors that feature naturally occurring tumor variation akin to that observed in human tumor populations. The goal is to identify and validate biomarkers that ultimately predict responsive versus non-responsive patient populations to guide clinical development.

**4:50 Appropriate Animals Models for Safety Assessment of Biologics**  
Timothy MacLachlan, Ph.D., Associate Director of Nonclinical Safety Assessment, Genzyme Corporation

The proper safety assessment of biopharmaceuticals has been an evolving process. While the paradigm set for small molecules was applied early, and some aspects have remained the same, other areas have required modification. The specificity of some biologics like monoclonal antibodies has necessitated study in higher order species. However, alternatives such as mice transgenic for the human target have proved useful, and at times, more accurate in risk assessment. Examples of these approaches will be discussed.

**5:20 Comparative Oncology Drug Development**  
Melissa C. Paoloni, DVM, DACVIM, Director, Comparative Oncology Program, National Institutes of Health, National Cancer Institute

Comparative oncology is a model system to evaluate novel drugs, devices, biologics and imaging strategies in pet dogs with cancer to help inform their development for human cancer patients. Although much of this effort is preclinical, it also applies to agents that are already “first in man.” The goal behind this is to garner information to help make informed go and no-go decisions to drive human oncology clinical trial design by answering questions about PK, PD, schedule, regime, dose, toxicity and clinical outcome (to just name a few). It has been well recognized and utilized by many within the pharmaceutical industry. It also has the ability to pilot personalized medicine approaches—a key to the future of oncology drug development.

**5:50 Close of Day**

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**FRIDAY, FEBRUARY 5**

**TRANSLATIONAL INFORMATICS – HOW FAR HAVE WE COME?**

**8:30 AM Chairperson’s Opening Remarks**  
Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.

**8:35 Implementing a Translational Biomarker Strategy to Reduce Attrition in Drug Development**  
Irina Antonijevic, M.D. Ph.D., Director, Translational Research, Biological Research, Lundbeck Research, Inc. USA

Early efforts towards the discovery of molecular biomarkers for CNS disorders are encouraging. However, confirmation, and ultimately validation of such biomarkers is dependent on state-of-the-art bioinformatics analyses as well as assay development. These prerequisites will ensure identification of biomarkers that are reproducible and hence of clinical relevance.

**9:05 High Content Mining of Disease Biomarkers**  
Jake Chen, Ph.D., Assistant Professor, Informatics & Computer Science, Indiana University School of Informatics; Director, Indiana Center for Systems Biology and Personalized Medicine, Indiana University - Purdue University Indianapolis; Founder, MedesLinx, Inc.

To facilitate the interpretation of raw Omics data into detailed disease-specific knowledge of candidate biomarkers, we developed a “high-content biomarker mining” software system. The system can help manage and correlate molecular functions, molecular connectivity, biological pathways, and literature information. Its application into the current biomarker development process will help improve the success rate and quality of candidate biomarkers.

Eric Schadt, Ph.D., Chief Scientific Officer, Pacific Biosciences

While there has been an explosion of technologies that enable more comprehensive characterizations of complex biological processes like common human diseases, we are still unable to glimpse a large enough fraction of the biology of these systems to build models that are predictive enough to achieve clinical utility. However, with a new wave of technologies on the horizon, providing for the capability to examine the activity of single molecules real time, we will soon be capable of generating the right scale and diversity of data (DNA sequence, RNA sequence, real time monitoring of mRNA translation, full characterizations of base modifications in genomes and transcriptomes) at low cost to dramatically enhance the construction of models for common human diseases that achieve clinical utility. I will cover the single molecule real time (SMRT) technologies from Pacific Biosciences and how these technologies will revolutionize our ability to characterize living systems, and then present a number of integrative biology approaches to taking the types of data SMRT technologies will generate to get at predictive models of disease that can be used to drive the identification and validation of drug targets and biomarkers.

**10:05 Automating Biomarker Discovery and Qualification; Capturing Hypothesis, Analysis and IP**  
Jonathan Sheldon, Ph.D., Director of Translational Medicine, IDBS

Long lists of un-annotated proteins and genes are not a sufficient end point for ‘omics analysis, they need to be annotated with data from many public and proprietary sources. IDBS provide solutions to not only automate the discovery and subsequent annotation of biomarker results, but to capture each step of the experimental set up, data capture, and analysis in a compliant manner.

**10:20 Coffee Break**

**TRANSLATIONAL INFORMATICS – HOW FAR HAVE WE COME? (CONTINUED)**

**11:00 Profiling Patients to Drive Biomarker Development**  
N. R. Nirmala, Ph.D., Director, Biomarker Analysis and Informatics Unit, Translational Sciences, Novartis Institutes of Biomedical Research

Gene expression profiling is one of the key ways in which a genome-wide view of a patient’s response to drug treatment can be obtained. Such a molecular level view can provide strategies for customized therapies in many contexts. In this talk, the opportunities and challenges that this technology presents will be discussed with a couple of case studies. Extension of this approach to other technologies will also be presented in the context of biomarker development.
11:30 Panel: Informatics at R&D Interphases
Moderator: Pearl S. Huang, Ph.D., Integrator, Oncology Franchise, Research & Early Development, Merck & Co.
- Linking clinical outcome with molecular data: filling the gaps
- Capturing uniform clinical language for outcomes
- Compatible and user-friendly data systems—can one size fit all?
- Disease cohorts—how many, how big, what is acceptable quality

12:00 PM Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

CASE STUDIES ON SUCCESSFUL ORGANIZATIONAL COLLABORATIONS AND SYSTEM APPROACHES

1:00 Chairperson’s Remarks
William B. Mattes, Ph.D., DABT; Former Executive Director, Predictive Safety Testing Consortium, Critical Path Institute

1:05 Taking personalized medicine to the next level- The critical Interface between Translational Medicine and Molecular Diagnostics
Lise Kjems, M.D. Ph.D., Executive Director, Global Program DiagnosticDirector, Molecular Diagnostics Novartis
Translational Medicine has reformed our drug development paradigm. Early clinical profiling of New Molecular Entities can enable identification of subsets of patients with a unique risk/benefit profile. In this talk, the opportunities and challenges related to this identification will be discussed. The role of Molecular Diagnostics is critical in developing and integrating novel patient treatment algorithms

1:35 Fostering Collaborations between Biotech and Academics to Speed Translational Medicine
Thomas Ichim, Ph.D., CEO, MediStem Labs, Inc.

2:05 Commercial Collaborations and other Approaches to Direct Academic Cancer Research towards Clinical Outcomes
Clive Stanway, Ph.D., CSO, Cancer Research Technology Ltd., Wolfson Institute for Biomedical Research
Cancer Research Technology (CRT) is the development and commercialization arm of Cancer Research UK (CR-UK) which has an annual science spend in excess of $500M. CRT works with CR-UK through multiple tracks to drive translational research including dedicated industry experienced, peer-reviewed funding for managed research in the PI’s laboratory or in collaboration with focused drug discovery research groups around the UK. Specific examples and outcomes of this strategy will be presented with some discussion of CRT’s flexible and creative approach to partnerships.

2:35 Development of Combination Therapies for Multiple Sclerosis Using Systems Level Informatics
Frederic S. Young, Ph.D., Chief Scientist, Vicus Therapeutics
We start with a multilevel systems physiology model that combines metabolomic analysis with integrated physiological analysis. The model is used to define a set of systems informatic features of ontogeny, phylogeny, homeostasis, and repair that distinguishes the disease state from homeostasis. We describe our use of this systems informatic signature as an algorithm for the development of combination therapies for multiple sclerosis.

3:05 Close of Conference
Mr. Crowley’s emotion-packed presentation will focus on his personal struggle to find a cure for Pompe disease, a rare and fatal illness that is caused by a defective or missing enzyme. Pompe disease affects fewer than 10,000 people worldwide, including Mr. Crowley’s two small children.

Mr. Crowley, a Harvard-educated businessman, created and built a pharmaceutical company devoted expressly to finding a cure for the disease. He will detail his journey through the labyrinth of scientific and business fronts, which lead up to a first-round clinical trial.

As information technologies and life sciences continue to converge, new business opportunities and challenges will arise for the field of diagnostics and beyond. This keynote lecture will describe this current pivotal point in brain evolution and how we can harness the new technology and lifestyle choices to improve memory and brain function so we can live better and longer.
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For the first time ever, the Molecular Medicine Tri-Conference will feature a New Product Showcase Pavilion. The New Product Showcase Pavilion is the place for exhibitors to introduce and promote their new product to conference attendees. CHI will promote the New Product Showcase Pavilion in our pre-show promotions, on our website, as well as on-site.

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