



Announcing the 2016
Keystone Symposia conference on:

Modern Phenotypic Drug Discovery: Defining the Path Forward

April 2–6, 2016

Big Sky Resort | Big Sky, Montana | USA

Scientific Organizers:

Jonathan A. Lee, Ellen L. Berg and Eugene C. Butcher

This meeting uniquely provides this interdisciplinary environment and is a particularly attractive venue given the increasing role of academia in drug discovery research. The conference will be a forum for the global Phenotypic Drug Discovery (PDD) research community in which scientists from diverse institutions and scientific disciplines can meet to share/discuss/debate topics related to advantages/disadvantages of PDD and how PDD can complement targeted approaches. Significantly, the conference will provide a much needed forum for the growing interest in PDD, and can conceivably become the cornerstone of the global scientific movement for the reintroduction of functional biology/physiology-driven pharmaceutical research.

Session Topics:

- Why Revisit PDD?
- PDD: Recent Clinical Successes
- Challenges to Modern PDD/Lessons Learned
- Workshop and Panel: PDD – Trends and Roadblocks
- New Biological Models
- Translational PDD: Physiologically Relevant Models
- Target/MOA Identification/Deconvolution
- Network Biology: New Technologies and Informatics
- Workshop and Panel: Identification of Molecular Targets and Biological “Mechanisms of Action”: How Much is Enough?
- Biologically Relevant Chemical Diversity

CONFIRMED SPEAKERS

(as of May 18, 2015):

Andreas Bender
Ellen L. Berg
Andrea Califano
Anne E. Carpenter
Benjamin F. Cravatt III
Eugen Dhimolea
Lars G. J. Hammarström
Jeremy Jenkins
Salman Khetani
Susan Lindquist
Nick A. Meanwell
Mark Mercola
John Moffat
Bernard H. Munos
Garry P. Nolan
Rebecca M. Pruss
Michael S. Saporito
Monica Schenone
Peter G. Schultz
David C. Swinney
Jon Tinsley
Herbert Waldmann
Janet Woodcock



Submitting an abstract is a great way of participating in the conference through poster presentation and possible selection for a short talk.

Scholarship & Discounted Abstract Deadline: Dec 2, 2015

Abstract Deadline: Jan 5, 2016

Discounted Registration Deadline: Feb 2, 2016

For additional details, visit www.keystonesymposia.org/16D1.

KEYSTONE SYMPOSIA™
on Molecular and Cellular Biology
Accelerating Life Science Discovery

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Sponsored by Abide Therapeutics Inc., Janssen R&D: Pharmaceutical Companies of Johnson & Johnson, Merck & Co., Inc., Pfizer Inc., Takeda Pharmaceutical Company Limited and Vertex Pharmaceuticals Incorporated

Abstract & Scholarship Deadline: December 2, 2015 / Abstract Deadline: January 5, 2016 / Discounted Registration Deadline: February 2, 2016

SATURDAY, APRIL 2

Arrival and Registration

SUNDAY, APRIL 3

Keynote Address

***Eugene C. Butcher**, Stanford University, USA

David C. Swinney, iRND3, Institute for Rare and Neglected Diseases, USA

The Value of Phenotypic Assays to Drug Discovery: Historical Perspective

Why Revisit PDD?

***Eugene C. Butcher**, Stanford University, USA

John Moffat, Genentech, Inc., USA

Phenotypic and Targeted Drug Discovery Are Complimentary Strategies

Bernard H. Munos, FasterCures, USA

Pharma Research Productivity: A New Perspective on Phenotypic vs. Target-Based Screening

PDD: Recent Clinical Successes

***Ellen L. Berg**, Alto Predict LLC, USA

Nicholas A. Meanwell, Bristol-Myers Squibb, USA

Phenotypic Screening for Antiviral Agents

Rebecca M. Pruss, Windhover Biomed, France

Maintenance of Motor Function in Spinal Muscular Atrophy by Olesoxime: A Recent Clinical Success for Phenotypic Drug Discovery

Jon Tinsley, Summit Therapeutics, UK

Modulators of Utrophin Production for the Treatment of Duchenne Muscular Dystrophy

Veronica Soloveva, USAMRIID, USA

Short Talk: Identification and Characterization of the Antiviral Activity of GS-5734 using Phenotypic Approaches

Poster Session 1

MONDAY, APRIL 4

Challenges to Modern PDD/Lessons Learned

***Jonathan A. Lee**, PDD4Patients LLC, USA

Neil Oliver Carragher, University of Edinburgh, Scotland
Accelerating PDD through Integrating MOA Profiling with Agile Chemical Design: Shifting the Bottlenecks from Target Deconvolution to Disease Relevance

Ellen L. Berg, Alto Predict LLC, USA

Phenotypic Chemical Biology for Predicting Efficacy and Safety

Fabien Vincent, Pfizer, USA

Lessons Learned in Phenotypic Screening

Michael R. Jackson, SBP Medical Discovery Institute, USA

Phenotypic Screening of Patient Derived Cells: Balancing Throughput with Relevance

Bridget Wagner, Broad Institute, USA

Short Talk: Small-Molecule Stimulation of Human Beta-Cell Proliferation

Panel 1: PDD Trends and Roadblocks

***David C. Swinney**, iRND3, Institute for Rare and Neglected Diseases, USA

Ellen L. Berg, Alto Predict LLC, USA

Jonathan A. Lee, PDD4Patients LLC, USA

Bernard H. Munos, FasterCures, USA

Fabien Vincent, Pfizer, USA

Neil Oliver Carragher, University of Edinburgh, Scotland

Michael R. Jackson, SBP Medical Discovery Institute, USA

New Biological Models

Eugen Dhimolea, Dana Farber Cancer Institute, Harvard Medical School, USA

Engineering 3-D in vitro Tumor Models of Antineoplastic Drug Resistance

Salman Khetani, University of Illinois at Chicago, USA

Microengineered Human Liver Models for Drug Screening and Disease Modeling

Sandy Hinckley, Sanford Burnham Prebys Medical Discovery Institute, USA

Short Talk: Phenotypic Screening of Neuronal Networks using High-throughput Multielectrode Arrays

Deborah Greene Nguyen, Organovo, USA

Short Talk: Bioprinted Three-dimensional (3D) Human Tissues for Toxicology and Disease Modeling

David Gerber, Galenea Corp., USA

Short Talk: Targeting Synaptic Function for CNS Drug Discovery

Poster Session 2

TUESDAY, APRIL 5

Translational PDD: Physiologically Relevant Models

***Fabien Vincent**, Pfizer, USA

Andrew Reaume, Melior Discovery, Inc., USA

Case Studies and Overall Learnings from 10 Years of Phenotypic Screening with a Broad-Based Panel of In Vivo Models

Mark Mercola, Stanford University, USA

Functional and Chemical Genomics to Decipher Heart Failure Mechanisms

Tom Large, Blue Oak Pharmaceuticals, USA

Discovery of Novel Psychiatric Drugs using a Target-agnostic, Systems Neurobiology Strategy

JASON PTACEK, Ionpath, Inc, USA

Short Talk: Phenotypic Human Primary Cell Based Tumor Microenvironment Models for Evaluation of Drug Combinations for Immune Oncology

David A. Mann, Cellular Dynamics International Inc., USA

Short Talk: Relevant Phenotypic Drug Discovery: Innate, Induced and Engineered Human iPSC Models

Denise V. Barrault, University of Edinburgh, UK

Short Talk

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NIH Common Fund Program

Aaron Pawlyk, NIDDK, National Institutes of Health, USA
In Search of Phenotypes to Illuminate the Druggable Genome

Biologically Relevant Chemical Diversity

***Paul Clemons**, Broad Institute of Harvard and MIT, USA
Chemical Diversity Expressed through Small-Molecule Performance: Diverse Readouts, Phenotypes and Genetic Determinants

Herbert Waldmann, Max Planck Institute for Molecular Physiology, Germany
Natural Product Inspired Compound Collections: Evolutionary Principle, Chemical Synthesis, Phenotypic Screening and Target Identification

Nicole E. Bodycombe, Pfizer, USA
Compound Subsets in Phenotypic Screening

Andrea Galmozzi, The Scripps Research Institute, USA
Short Talk: Phenotypic Screening of Fully Functionalized Fragment-Based Chemical Probes Reveals PGRMC2 As A New Regulator of Adipocyte Physiology of Therapeutic Potential for Obesity-Linked Diabetes

Poster Session 3

WEDNESDAY, APRIL 6

Keynote Address

***John Moffat**, Genentech, Inc., USA

Enrique Saez, The Scripps Research Institute, USA
Chemical Proteomic Strategies to Advance Phenotypic Screening

Target/MOA Identification/Deconvolution

***John Moffat**, Genentech, Inc., USA

Monica Schenone, Pfizer, USA
Target Identification and Mechanism of Action in Drug Discovery

Jeremy Jenkins, Novartis Institutes for BioMedical Research, USA
Systems Approaches to Elucidating Compound-Effect Pathways

Andreas Bender, University of Cambridge, UK
Towards Understanding Phenotypic Readouts - Utilizing Multiple Types of Chemical and Biological Data to Rationalize Compound Action

Vladimir Chupakhin, Janssen Pharmaceuticals, Belgium
Short Talk: The New Approaches for a Computational Target Deconvolution of the Phenotypic Hits

Susanne Swalley, Biogen, USA
Short Talk: Phenotypic Drug Discovery in SMA: Parallel Efforts in Preclinical Development and Target Identification

Panel 2: Identification of Molecular Targets and Biological "Mechanisms of Action": How Much is Enough?

***Eugene C. Butcher**, Stanford University, USA

Susanne Swalley, Biogen, USA

Tom Large, Blue Oak Pharmaceuticals, USA

Marco Prunotto, F. Hoffmann-La Roche Ltd., Switzerland

Nicholas A. Meanwell, Bristol-Myers Squibb, USA

Rebecca M. Pruss, Windhover Biomed, France

Network Biology: New Technologies and Informatics

Andrea Califano, Columbia University, USA
Systematic Elucidation of Druggable Dependencies in Human Malignancies: A New Take on Precision Medicine

Sean Bendall, Stanford University, USA
Revealing Hidden Behavior One Cell at a Time

Anne E. Carpenter, Broad Institute of Harvard and MIT, USA
Increasing the Content of High-Content Phenotypic Assays: Targeting Diseases and Characterizing Compounds via Cell Painting and Image-Based Profiling

***Marco Prunotto**, F. Hoffmann-La Roche Ltd., Switzerland
Short Talk: Application of Molecular Phenotype to Phenotypic Drug Discovery: Results of a PoC Study

THURSDAY, APRIL 7

Departure