

# KEYSTONE SYMPOSIA

on Molecular and Cellular Biology

## Phenotypic Drug Discovery: Recent Advances and Insights from Chemical and Systems Biology (C1)

March 3-7, 2019 • Beaver Run Resort • Breckenridge, Colorado, USA

Scientific Organizers: Mark Mercola, Fabien Vincent, Monica Schenone and Arsenio Nueda

Supported by the Directors' Fund

Abstract & Scholarship Deadline: October 30, 2018 / Abstract Deadline: December 4, 2018 / Discounted Registration Deadline: January 11, 2019

### SUNDAY, MARCH 3

#### Arrival and Registration

### MONDAY, MARCH 4

#### Keynote Address

**James E. Bradner**, Novartis Institutes for BioMedical Research, USA  
*PDD, New Approach to Discovering Therapeutics*

#### Clinical Success Stories

**Yoshihiro Watanabe**, Kanazawa University, Japan  
*Discovery of JTP-74057/GSK1120212/Trametinib as a MEK-Inhibitor able to alter Phosphorylation State of MEK*

**Fred Van Goor**, Vertex Pharmaceuticals Incorporated, USA  
*Combination Pharmacology to Treat the Underlying Cause of the Majority of People with Cystic Fibrosis*

**John Tallarico**, Novartis Institutes of Biomedical Research, USA  
*Novel Therapies from Phenotypic Screening*

#### Short Talk(s) Chosen from Abstracts

#### Workshop 1: Safety De-risking

\***Fabien Vincent**, Pfizer, USA

#### Short Talks Chosen from Abstracts

#### New Opportunities and Under-Represented Indications

**Lucienne V. Ronco†**, Fulcrum Therapeutics, USA  
*Talk Title to be Announced*

**Clemens R. Scherzer**, Harvard Medical School, Brigham & Women's Hospital, USA  
*Turning Bits and Base Pairs into Precision Neurology*

**Scott S. Walker**, Merck Research Laboratories, USA  
*Large-scale Antibacterial Phenotypic Screening and Systematic Hit Triage Reveal Novel Targets and Novel Inhibitors of Established Targets*

#### Short Talk Chosen from Abstracts

#### Poster Session 1

### TUESDAY, MARCH 5

#### Chemical Biology and Novel Molecular Mechanisms of Action

**Maurizio Pellecchia**, University of California, Riverside, USA  
*NMR-based Fragment Screening for Oncology*

**Laura L. Kiessling**, Massachusetts Institute of Technology, USA  
*Glycans in Health and Disease*

**Razvan L. Cornea**, University of Minnesota, USA  
*Targeting the Pathological Leak of a Calcium Channel for Heart Failure Therapeutics*

**Angela Koehler**, Massachusetts Institute of Technology, USA  
*Chemical Probes of Transcription*

#### Short Talk(s) Chosen from Abstracts

#### Project Prosecution and Pre-clinical Advancement

**Arsenio Nueda**, Almirall S.A., Spain

*Harnessing the Power of Phenotypic Screening to Generate Novel Starting Points for Drug Discovery*

**Chun-wa Chung**, GlaxoSmithKline, UK

*Linking Differential Molecular Mode of Action to Distinct Pharmacology*

**Derek Lowe**, Novartis Institutes for Biomedical Research, USA

*Advancement of Phenotypic Hits*

#### Short Talk Chosen from Abstracts

#### Poster Session 2

### WEDNESDAY, MARCH 6

#### Physiologically Complex and Realistic Disease Assays

**Rick Livesey**, University of Cambridge, UK

*Target Identification and Phenotypic Screening in Stem Cell Models of Alzheimer's Disease*

**Randall T. Peterson**, University of Utah, USA

*Drug Discovery using Zebrafish*

**Mark Mercola**, Stanford University, USA

*High Throughput Kinetic Imaging of iPSC-Derived Cardiomyocytes: Bringing the Patient to the Early Stages of Drug Discovery*

#### Speaker to be Announced

#### Short Talk(s) Chosen from Abstracts

#### Workshop 2: Clinical, Regulatory Case Studies with PDD Project Prosecution

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

#### Short Talks Chosen from Abstracts

#### Functional Genomics and Systems Approaches

#### Speaker to be Announced

**Thomas A. Wynn**, Pfizer, USA

*siRNA and CRISPR Screening in Primary Human Cells for Autoimmune and Fibrotic Diseases*

**Giulio Superti-Furga**, Research Center for Molecular Medicine of the Austrian Academy of Sciences, Austria

*Systems Pharmacology as an Approach to Network Medicine*

#### Short Talk Chosen from Abstracts

#### Poster Session 3

### THURSDAY, MARCH 7

#### Keynote Address

**Benjamin F. Cravatt III**, The Scripps Research Institute, USA

*Activity-based Proteomics – Target and Ligand Discovery on a Global Scale*

#### Compound Profiling and Repositioning

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**Christopher A. Lipinski**, Melior Discovery, Inc., USA

*High Throughput in vivo Phenotypic Screening for Drug Repurposing: Discovery of MLR-1023 a Novel insulin Sensitizer and Novel Lyn Kinase Stimulant with Clinical Proof of Concept*

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

*Molecular Phenotyping Combines Molecular Information, Biological Relevance, and Patient Data to Improve Productivity of Early Drug Discovery*

**Umut Ozcan**, Boston Children's Hospital, Harvard Medical School, USA

*Withaferin A is a Leptin Sensitizer with Strong Antidiabetic Properties in Mice*

### Short Talk(s) Chosen from Abstracts

#### Target Identification / Deconvolution

**Dyann F. Wirth**, Harvard School of Public Health, USA

*Target ID for Malaria*

**Bridget Wagner**, Broad Institute, USA

*The Importance of Phenotypic Screening in Identifying New Targets for Disease Therapeutics*

**Herbert Waldmann**, Max Planck Institute for Molecular Physiology, Germany

*Chemotype - Phenotype - Target*

#### Meeting Wrap-Up: Outcomes and Future Directions (Organizers)

#### FRIDAY, MARCH 8

#### Departure