

**ITMAT 10<sup>th</sup> Annual International Symposium  
Monday and Tuesday, October 12-13, 2015  
Perelman School of Medicine, University of Pennsylvania  
Smilow Center for Translational Research (SCTR)  
The Arthur Rubenstein Auditorium  
3400 Civic Center Boulevard, Philadelphia, PA**

***Systems Pharmacology and Translational Therapeutics***

Monday, October 12

DAY 1

8:15 Registration and breakfast, SCTR Lobby

9:15 Introduction  
Jonathan A. Epstein, MD, Executive Vice Dean and Chief Scientific Officer,  
Perelman School of Medicine, University of Pennsylvania

*Session 1: Translational Science at home and abroad.*  
Session Chairs: Beatrice H. Hahn, MD, Professor, Departments of Medicine and Microbiology, Perelman School of Medicine, University of Pennsylvania and Sir Keith Peters, FRCP, FRS, FMedSci, Emeritus Regius Professor of Physic, University of Cambridge, Consultant in Clinical Science and Translational Medicine to the Francis Crick Institute and Senior Consultant in R&D to GlaxoSmithKline

9:30 ***Role of FDA in Stimulating Translational Science***  
Robert M. Califf, MD  
Deputy Commissioner for Medical Products and Tobacco US Food and Drug Administration

10:00 ***The German Centre for Cardiovascular Research – a new structure to promote translational research***  
Thomas Eschenhagen, MD, Professor of Pharmacology, Department of Experimental Pharmacology and Toxicology, University Medical Center Hamburg-Eppendorf, Germany

10.30 ***Academia, government and industry: lessons learned in translational medicine***  
Elias Zerhouni, MD, President, Global R&D, Sanofi

11:00 COFFEE

**Session 2: *The evolving search for Drug Targets - 1***

Session Chairs: Joan M. O'Brien, MD, William F. Norris and George E. deSchweinitz, Professor, Chairman, Department of Ophthalmology, Director, Scheie Eye Institute, University of Pennsylvania and Lynn M. Schuchter, MD, C. Willard Robinson Professor of Hematology/Oncology Chief, Division of Hematology/Oncology Program Leader, Melanoma & Cutaneous Malignancies Program, Abramson Cancer Center of the University of Pennsylvania

11.30 ***Nuclear Receptors: Feast, Famine and Physiology***

Ronald M. Evans, PhD, Professor, The Salk Institute for Biological Studies, Investigator, Howard Hughes Medical Institute, March of Dimes Chair in Molecular and Developmental Biology

12:00 **Combinatorial pharmacogenetics**

Jason H. Moore, PhD, Professor and Director, Penn Institute for Biomedical Informatics, University of Pennsylvania

12.30 LUNCH

Poster Session: *Science Translational Medicine* Associate Scientific Advisors will present their research in a two-day poster session held in the breakfast/lunch area. The scientists will be at their posters during the second half of the lunch break on Monday and Tuesday. The poster sessions as well as the attendance of the *Science Translational Medicine* Associate Advisors at the ITMAT Symposium were made possible by a generous grant from Sanofi and funding from the American Association for the Advancement of Science.

**Session 3: *The evolving search for Drug Targets - 2***

Session Chairs: Thomas P. Cappola, MD, Chief, Division of Cardiovascular Medicine, University of Pennsylvania Perelman School of Medicine and Charles S. Abrams, MD, Vice Chair for Research & Chief Scientific Officer, Professor of Medicine, Chief, Non-Malignant Hematology, Director, Blood Center at PENN/CHOP, University of Pennsylvania School of Medicine

1:30 ***Non-coding RNAs: Therapeutic targets in cardiovascular disease?***

Stefanie Dimmeler, PhD, Institute of Cardiovascular Regeneration, Goethe University Frankfurt

- 2:00      ***iPSCs for Accelerating Drug Discovery Process***  
Joseph C. Wu, MD, PhD, Director, Stanford Cardiovascular Institute, Professor, Department of Medicine (Cardiology) and Department of Radiology (Molecular Imaging Program), Stanford University School of Medicine
- 2.30      ***Bringing Genetics & Genomics to the Clinic: What, Where, and When***  
David Ginsburg, MD, James V. Neel Distinguished University Professor, Departments of Internal Medicine, Human Genetics and Pediatrics and Communicable Diseases, Howard Hughes Medical Institute, University of Michigan Medical School
- 3:00      ***Stem and progenitor dysfunction in human trisomies***  
Irene Roberts, MD, Professor of Paediatric Haematology, Department of Paediatrics and Weatherall Institute of Molecular Medicine, University of Oxford
- 3.30      COFFEE
- Session 4:      ***Big data and the Phenome; What you see is what you get***  
Session Chairs: John Hogenesch, PhD, Ohio Eminent Scholar, Professor of Molecular and Cellular Physiology and Pediatrics, University of Cincinnati College of Medicine, Cincinnati Children's Hospital Medical Center and Maja Bucan, PhD, Professor of Genetics, The Perelman School of Medicine at the University of Pennsylvania
- 4:00      ***Bogosity in Big Data***  
Isaac Kohane, MD, PhD, Chair, Department of Biomedical Informatics, Harvard Medical School
- 4.30      ***Optical deconstruction of fully-assembled biological systems***  
Karl Deisseroth MD, PhD, D. H. Chen Professor of Bioengineering and of Psychiatry and Behavioral Sciences, Stanford University
- 5:00      ***Big Data in Translational Research***  
Ewan Birney, PhD, Associate Director, EBI, UK
- 7.00      Reception and Dinner: The College of Physicians of Philadelphia, 19 South 22nd Street, Philadelphia  
Dinner Speaker: Two time Poet Laureate, Billy Collins

*Transportation will be provided to and from the dinner. Buses will depart from the front of the Perelman Center for Advanced Medicine (circular driveway in front of building that connects to the Smilow Center for Translational Research) at 6:40PM and will return to the Hilton Inn at Penn at approximately 9:00 PM.*

*Speakers and chairs may take a shuttle bus from the Hilton Inn at Penn, Walnut Street entrance, departing at 6:40PM, returning to the hotel at approximately 9:00 PM.*

Tuesday, October 13  
Day 2

8:30 Registration and breakfast SCTR Lobby

**Session 5: *Systems Approaches to Pharmacobiology***

Session Chairs: James Eberwine, PhD, Elmer Holmes Bobst Professor, Co-Director of the PENN Program in Single Cell Biology, Dept. of Systems Pharmacology and Translational Therapeutics, University of Pennsylvania Perelman School of Medicine and Junhyong Kim, PhD, Patricia M. Williams Term Professor in Biology; Co-Director, Penn Genomics Institute; Adjunct Professor, Computer and Information Science, University of Pennsylvania

9.00 ***Leveraging wisdom of crowds to evolve more predictive models of disease***  
Eric E. Schadt, PhD, Director, Icahn Institute for Genomics at Multiscale Biology at Mount Sinai

9:30 ***Pharmacology at a single-cell level***  
Peter Sorger, PhD, Otto Kraymer Professor of Systems Pharmacology, Department of Systems Biology, Harvard Medical School

10:00 ***Systematic elucidation of druggable dependencies in human malignancies: a new take on precision medicine***  
Andrea Califano, PhD, Chair, Department of Systems Biology, Columbia University

10:30 COFFEE

**Session 6: *Parsing variability in the pathogenesis of disease***

Session Chairs: Des Fitzgerald, MD, Professor of Molecular Medicine, Vice President for Health Affairs, University College Dublin, and Chief Academic Officer, Ireland East Healthcare and Vladimir Muzykantov, MD, PhD, Professor of Pharmacology and Medicine, Vice-Chair, Department of Systems Pharmacology and Translational Therapeutics, Director, Center for Targeted Therapeutics & Translational Nanomedicine, Perelman School of Medicine, University of Pennsylvania

11.00 ***Genome Engineering with CRISPR-Cas9: From Mechanism to Therapy***  
Jacob Corn, PhD, Director, Innovative Genomics Initiative at UC Berkeley/UCSF

11:30 ***DNA nanotech and super-resolution imaging***  
Peng Yin, PhD, Associate Professor, Wyss Institute for Biologically Inspired Engineering, Harvard University, Department of Systems Biology, Harvard Medical School

12.00 ***Transmission of Misfolded Proteins in Neurodegenerative Disorders: A Common Mechanism of Disease Progression***  
Virginia M.-Y. Lee, PhD, Professor of Pathology and Laboratory Medicine, Director, The Center for Neurodegenerative Disease Research at the Perelman School of Medicine of the University of Pennsylvania

12:30 LUNCH and Poster Session

*Session 7: Emerging therapeutic paradigms in Cancer*

Session Chairs: Daniel J Rader, MD, Associate Director, ITMAT, Chair, Dept. of Genetics, Perelman School of Medicine, University of Pennsylvania and Tom Curran, PhD, FRS, Deputy Scientific Director, The Children's Hospital of Philadelphia Research Institute, Professor of Pathology and Laboratory Medicine, Cell & Developmental Biology, Perelman School of Medicine, Associate Director Translational Genomics, University of Pennsylvania

1:30 ***Signaling regulation of Beclin 1 in Autophagy and Tumorigenesis***  
Beth Levine, MD, Howard Hughes Medical Institute, Professor, Internal Medicine and Microbiology, Charles Cameron Sprague Distinguished Chair in Biomedical Science, Director, Center for Autophagy Research, UT Southwestern Medical Center

2.00 ***PD-1 pathway blockade in cancer therapy: a common denominator approach to precision medicine***  
Suzanne L. Topalian, MD, Professor, Surgery and Oncology, Johns Hopkins University School of Medicine, Director, Melanoma Program, Sidney Kimmel Comprehensive Cancer Center

2:30 **BREAK**

2:45 ***Targeting Immunity with Bioelectronics***  
Kevin J. Tracey, MD, President & CEO, Feinstein Institute for Medical Research, Professor of Neurosurgery and Molecular Medicine Associate Dean, Research Hofstra North Shore-LIJ School of Medicine

3:15 ***Designing CARs for cancer therapy***  
Carl June, MD, Richard W. Vague Professor in Immunotherapy, Perelman School of Medicine, University of Pennsylvania

3:45 ***Concluding Remarks***  
Garret A. FitzGerald, MD, FRS, Perelman School of Medicine, University of Pennsylvania