

ITMAT 9th Annual International Symposium
Monday and Tuesday, October 13-14, 2014
Perelman School of Medicine, University of Pennsylvania
Smilow Center for Translational Research (SCTR)
The Arthur Rubenstein Auditorium
3400 Civic Center Boulevard, Philadelphia, PA

Progress in Translational Science: Emerging Therapeutic Modalities

Monday, October 13

DAY 1

8:15 **Registration and breakfast, SCTR Lobby**

9:15 **Introduction**
J. Larry Jameson, MD, PhD, Executive Vice President, University of Pennsylvania for the Health System, and Dean, Perelman School of Medicine, University of Pennsylvania

Session 1.1: Challenges and Opportunities in Translational Research

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 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 ***Opportunity for Translation: a European Perspective***
Sir Leszek Borysiewicz, FRS FRCP FMedSci, The Vice-Chancellor, University of Cambridge

10.00 ***Clinical Development Success Rates – New and Updated Analysis***
Michael Hay, Executive Vice President, Sagient Product Director, Sagient Research, Informa Business Information

10.30 COFFEE

Session 1.2: Challenges and Opportunities in Translational Research

Session Chairs: Daniel J Rader, MD, Associate Director, ITMAT, Chair, Dept. of Genetics, Perelman School of Medicine, University of Pennsylvania and Barry Collier, MD, Professor, Physician-in-Chief, Vice President for Medical Affairs, Rockefeller University

- 11.00 ***Current Controversies in Scientific Publishing***
Katrina Kelner, PhD, Editor, Science Translational Medicine (AAAS)
- 11.30 ***How to be successful in biotech without really trying***
Charles Weissmann, MD, PhD, Scripps Florida
- 12.00 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. *SciTM* thanks Sanofi, Novartis, and GlaxoSmithKline for their generous support.

Session 2: Stem Cell Therapeutics

Session Chairs: Beverly L. Davidson, PhD, Arthur V. Meigs Chair in Pediatrics, Director, Center for Cell and Molecular Therapy, Children's Hospital of Philadelphia and Emma A. Meagher, MD, Associate Vice Provost, Human Research, Associate Professor Medicine and Pharmacology, University of Pennsylvania

- 1.00 ***Discovering networks and diagnostics for pancreatic cancer progression***
Kenneth S. Zaret, PhD, Joseph Leidy Professor of Cell and Developmental Biology, Perelman School of Medicine, University of Pennsylvania
- 1.30 ***Studying neurodegenerative disease in a dish***
Clifford J. Woolf, MB, BCh, PhD, Director, F.M. Kirby Neurobiology Center and Program in Neurobiology, Boston Children's Hospital, Professor of Neurology and Neurobiology, Harvard Medical School
- 2.00 ***Direct lineage reprogramming towards the neural lineage***
Marius Wernig, MD, Assistant Professor, Stanford University
- 2.30 ***Stem cells in silence, action and cancer***
Elaine Fuchs, PhD, Rebecca C. Lancefield Professor, HHMI, The Rockefeller University
- 3.00 COFFEE

Session 3: Movement in Malaria

Session Chairs: Desmond Fitzgerald, MD, Professor of Molecular Medicine, University College Dublin and Sir Gordon Duff, PhD, FRCP, FRCPE, FMedSci, FRSE, St. Hilda's College, Oxford

- 3.30 ***Developing new drugs for malaria elimination and eradication***
Elizabeth Winzeler, PhD, Professor of Pediatrics, Division of Pharmacology and Drug Discovery, Director of Translational Research, UC Health Sciences Center for Immunology, Infection and Inflammation, University of California, San Diego, School of Medicine
- 4.00 ***Tackling malaria: New approaches to an ancient problem***
Manuel Llinás, PhD, Associate Professor, Pennsylvania State University, The Huck Institutes of the Life Sciences, Dept. of Biochemistry & Molecular Biology, Center for Infectious Disease Dynamics, Center for Malaria Research
- 4.30 ***Ape Reservoirs of Human Malaria***
Beatrice H. Hahn, MD, Professor, Departments of Medicine and Microbiology, Perelman School of Medicine, University of Pennsylvania
- 7.00 **Reception and Dinner: The College of Physicians of Philadelphia, 19 South 22nd Street, Philadelphia**
Dinner Speaker: Christopher P. Austin, MD, Director, National Center for Advancing Translational Sciences, NIH

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 9:30 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 9.30 PM Dinner

Tuesday, October 14 Day 2

- 9:00 Registration and breakfast SCTR Lobby

Session 4: Focus on the Single Cell

Session Chairs: 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 and Ellen Puré, PhD, Grace Lansing Lambert Professor of Biomedical Science and Chair, Department of Animal Biology, University of Pennsylvania, School of Veterinary Medicine

9.30 ***Single Cell Parables: Heterogeneity Informs Translational Considerations***

Jim Eberwine, PhD, Elmer Holmes Bobst Professor, Co-Director of the PENN Genome Frontiers Institute, Department of Pharmacology, Perelman School of Medicine, University of Pennsylvania

10.00 ***Single Cell Protein Analysis: Potential for impact on clinical research and clinical outcomes***

David R. Klug, PhD, Chair of the Imperial College Institute of Chemical Biology, Department of Chemistry, Imperial College

10:30 ***Metabolomics at the single-cell level***

Renato Zenobi, PhD, Department of Chemistry and Applied Biosciences, ETH Zurich

11.00 COFFEE

Session 5: Variability in Drug Response

Session Chairs: Professor Sir Rory Collins, FMedSci, FRCP, Clinical Trial Service Unit & Epidemiological Studies Unit, Nuffield Department of Population Health, University of Oxford, UK and Robert H. Vonderheide, MD, DPhil, Hanna Wise Professor of Cancer Research, Perelman School of Medicine, University of Pennsylvania

11.30 ***Applications of Precision Medicine to Address Inter-Individual Variability in Response***

D. Gary Gilliland, MD, PhD, Vice Dean and Vice President for Precision Medicine, John Morgan Professor of Medicine, Investigator, Abramson Family Cancer Research Institute, Perelman School of Medicine, University of Pennsylvania

12.00 ***The role of the microbiome in response to cancer therapy***

Romina Goldszmid, PhD, Staff Scientist, Laboratory of Experimental Immunology, Cancer and Inflammation Program, Center for Cancer Research, NCI, NIH

12.30 ***Why good drugs are sometimes bad for the liver – from man to mouse to computer***

Paul Watkins, MD, Hamner - University of North Carolina Institute for Drug Safety Sciences

1.00 LUNCH

Session 6: Translational Immunology

Session Chairs: Lee Marshall Nadler, MD, Dean for Clinical and Translational Research, Harvard Medical School and E. John Wherry, PhD, Director, Institute for Immunology, Associate Professor of Microbiology, Perelman School of Medicine, University of Pennsylvania

2.00 ***Mechanisms and Biomarkers of CD8 T Cell Exhaustion***
E. John Wherry, PhD, Associate Professor of Microbiology, Chair, Immunology Graduate Group, Director, Institute for Immunology, Perelman School of Medicine, University of Pennsylvania

2.30 ***Driving CARs for cancer: are we there yet?***
Carl H. June, MD, Richard W. Vague Professor in Immunotherapy, Program Director of Translational Research, Abramson Family Cancer Research Institute, Perelman School of Medicine, University of Pennsylvania

3.00 ***New Frontiers in Inflammation Research***
Luke O'Neill, PhD, Director, Trinity Biomedical Sciences Institute, School of Biochemistry and Immunology, Trinity College Dublin

3:30 ***Concluding Remarks***
Garret A. FitzGerald, MD, FRS, Perelman School of Medicine, University of Pennsylvania