

ITMAT 2018

Smilow Center for  
Translational Research  
University of Pennsylvania



A Sense of  
Momentum:  
Translational  
Science Comes  
of Age



**ITMAT 13<sup>th</sup> Annual International Symposium  
Monday and Tuesday, October 15-16, 2018  
Perelman School of Medicine, University of Pennsylvania  
Smilow Center for Translational Research (SCTR)  
The Arthur Rubenstein Auditorium  
3400 Civic Center Boulevard, Philadelphia, PA**

*A Sense of Momentum; Translational Science Comes of Age*

**Monday, October 15 (Day 1)**

8:00 AM Registration and Breakfast: Smilow Lobby

8:45 ***Introduction***  
Jonathan A. Epstein, MD, Executive Vice Dean and Chief Scientific Officer,  
Perelman School of Medicine, University of Pennsylvania

***Session 1: Data at Scale, Pathobiology and Precision Medicine***

Chairs: Sir Keith Peters, Emeritus Regius Professor of Physic, University of Cambridge and Marylyn D. Ritchie, PhD, Director, Center for Translational Bioinformatics, Institute for Biomedical Informatics (IBI), University of Pennsylvania

9:00 ***Improving Immunotherapy for Malignant Disease: Molecules, Models, and Clinical Research***  
Roger M. Perlmutter, MD, PhD, EVP and President, Merck Research Laboratories

9:30 ***Precision medicine meets Global health***  
Maha Reda Farhat, MD, CM, MSc, Assistant Professor of Biomedical Informatics, Harvard Medical School, Assistant Physician, Massachusetts General Hospital

10:00 ***Translating Big Data and Artificial Intelligence into Precision Medicine***  
Kang Zhang, MD, PhD, Founding Director, Institute for Genomic Medicine, Co-Director, Biomaterials and Tissue Engineering, Institute of Engineering in Medicine; Chief of Ophthalmic Genetics; Professor of Ophthalmology, Nanoengineering, Human Genetics, University of California San Diego

10:30 COFFEE: Smilow Lobby

**Session 2: Novel Therapeutic Technologies – Part 1: Gene Editing**

**Chairs:** Katherine L. Nathanson, MD, Deputy Director, Abramson Cancer Center, Perelman School of Medicine at the University of Pennsylvania and Jorge Henao-Mejia, MD, PhD, Assistant Professor of Pathology and Laboratory Medicine Perelman School of Medicine at the University of Pennsylvania

11:00 **Engineering Immunotherapies with Synthetic Biology**

Timothy K. Lu, MD, PhD, Associate Professor, Massachusetts Institute of Technology

11:30 **Gene Editing to Prevent and Cure Metabolic Disorders**

Kiran Musunuru, MD, PhD, MPH, Associate Professor of Cardiovascular Medicine and Genetics, Perelman School of Medicine at the University of Pennsylvania

12:00 LUNCH AND POSTER SESSION: Smilow Lobby

Poster Session: current and past Associate Scientific Advisors of *Science Translational Medicine* 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.

**Session 3: Novel Therapeutic Technologies – Part 2: Gene Therapy, RNAi and Brain Delivery**

**Chairs:** Daniel J. Rader, MD, Seymour Gray Professor of Molecular Medicine; Chair, Department of Genetics; Associate Director, ITMAT, Perelman School of Medicine at the University of Pennsylvania and Vladimir Muzykantov, MD, PhD, Professor and Vice Chair of Systems Pharmacology and Translational Therapeutics and Medicine, Co-Director, Center for Translational Therapeutics and Translational Nanomedicine (CT<sup>3</sup>N)

1:00 **Turning Genes into Medicine**

Katherine A. High, MD  
President and Head of R&D  
Spark Therapeutics

1:30 **Advancement of Investigational RNAi Therapeutics, a New Class of Innovative Medicines**

Greg Hinkle, PhD, Sr. Director, Research and Head of Bioinformatics, Alnylam Pharmaceuticals

2:00 **The MiNDS: Miniaturized Neural Drug Delivery System**

Canan Dagdeviren, PhD, Assistant Professor of Media Arts and Sciences, Material Science & Engineering, MIT Media Lab, Massachusetts Institute of Technology

2:30 COFFEE: Smilow Lobby

**Session 4: Novel Therapeutic Technologies – Part 3: Vaccines and Stem Cell Biology**

**Chairs:** Kenneth S. Zaret, PhD, Joseph Leidy Professor, Director, Institute for Regenerative Medicine, University of Pennsylvania and George Cotsarelis, MD, Milton B. Hartzell Professor and Chairman, Dermatology, University of Pennsylvania

3:00 ***Synthetic Nucleic Acid Technology for Prevention and Immunotherapy of Human Disease***

David B. Weiner, PhD, Director, Vaccine & Immunotherapy Center, The Wistar Institute,

3:30 ***Stem cells in Silence, Action and Cancer***

Elaine Fuchs, PhD, Investigator, Rebecca C. Lancefield Professor, HHMI, The Rockefeller University

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7:00 **Reception and Dinner: The College of Physicians of Philadelphia**

19 South 22nd Street, Philadelphia

Dinner Speaker: Sir Keith Peters, Emeritus Regius Professor of Physic, University of Cambridge

*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 9:00PM.*

*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 9:00PM.*

## **Tuesday, October 16 (Day 2)**

8:00 AM Registration and Breakfast: Smilow Lobby

### **Session 5: *Nutrients and Aging***

**Chairs:** Kathryn E. Wellen, PhD, Associate Professor, Department of Cancer Biology, University of Pennsylvania and  
Park F. Cho-Park MD, PhD, Assistant Professor, Department of Systems Pharmacology & Translational Therapeutics, University of Pennsylvania

8:30 ***Autophagy, Caloric Restriction Mimetics and Body Mass Control***

Guido Kroemer, MD, PhD, Professor, University of Paris Descartes

9:00 ***Fan the Flame, Feed the Fire***

Cornelia M. Weyand, MD, PhD, Professor of Medicine and Chief, Division of Immunology and Rheumatology, Stanford University

9:30 ***Insulin Signaling and Quality of Life with Age***

Coleen T. Murphy, Ph.D., Professor of Genomics and Molecular Biology at Princeton University

10:00 COFFEE: Smilow Lobby

### **Session 6: *Therapeutic Interventions Part 1: Correcting Mitochondrial Dysfunction***

**Chairs:** Carlo Patrono, MD, Catholic University School of Medicine, Rome, Italy and  
Douglas C. Wallace, PhD, Director, Center for Mitochondrial and Epigenomic Medicine (CMEM), Children's Hospital of Philadelphia

10:30 ***Can We Prevent Germline Transmission of Mitochondrial DNA Mutations?***

Mary Herbert, PhD, Professor, Newcastle University

11:00 ***Hypoxia as a Therapy for Mitochondrial Disease***

Vamsi K. Mootha, MD, Harvard Medical School

***Therapeutic Interventions Part 2: Checkpoint Blockade***

11:30 ***Cancer Immune Phenotype and Therapy***

Weiping Zou, MD, PhD, Professor of Immunology, Pathology, Biology, and Surgery, University of Michigan School of Medicine

12:00 ***The Immune Checkpoint/Stem Cell Partnership to Cure T1D***

Paolo Fiorina, MD, PhD, University of Milan

12:30 LUNCH AND POSTER SESSION: Smilow Lobby

**Session 7: *Insights into Autism***

**Chairs:** Raquel E. Gur, MD, PhD, The Karl and Linda Rickels Professor, Director, Lifespan Brain Institute, Children's Hospital of Philadelphia and Penn Medicine and Benjamin F. Voight, PhD, Associate Professor, Systems Pharmacology and Translational Therapeutics, Associate Professor, Genetics, Perelman School of Medicine, University of Pennsylvania

1:30 ***Genetic Dissection of Autism Spectrum Disorders: Back to Phenotypes***  
Maja Bućan, PhD, Professor of Genetics, Departments of Genetics and Psychiatry, Perelman School of Medicine, University of Pennsylvania

2:00 ***Disruption in the Pattern of Connections in Autism***  
Helen Barbas, PhD, Professor, Boston University and School of Medicine

2:30 ***Autism: Alterations of Social Brain Networks at Early Developmental Stages***  
Holger Franz Sperdin, PhD, Research Associate, Developmental Imaging and Psychopathology Laboratory, University of Geneva, Switzerland

3:00 COFFEE: Smilow Lobby

**Session 8: *Targeting the Inflammasome***

Chairs: Igor Brodsky, PhD, Associate Professor of Pathobiology, University of Pennsylvania School of Veterinary Medicine and Desmond Fitzgerald, MD, President, University of Limerick, Ireland

3:30 ***Humanized Mice as a Tool for Investigating the Role of the Inflammasome in Disease Pathogenesis***  
Beverly H. Koller, PhD, Associate Professor, Department of Genetics, UNC, Chapel Hill

4:00 ***Inflammation at the Crossroads of Diabetes and Obesity***  
Alan R. Saltiel, PhD, Professor and Director of the UC, San Diego Institute for Diabetes and Metabolic Health, UC, San Diego

4:30 ***Inflammation Control by Inflammasomes***  
Kate Schroder, PhD Deputy Director of the IMB Centre for Inflammation and Disease Research, Institute for Molecular Bioscience, The University of Queensland

5:00 ***Concluding Remarks***  
Garret A. FitzGerald, MD, FRS, University of Pennsylvania