

ITMAT 2020

Smilow Center for
Translational Research
University of Pennsylvania



Novel Therapeutics
and Democratization of
Access: Implementation
of Translational Science



**ITMAT 15th Annual International Symposium
Monday and Tuesday, October 12-13, 2020
Perelman School of Medicine, University of Pennsylvania
Smilow Center for Translational Research (SCTR)
The Arthur Rubenstein Auditorium
3400 Civic Center Boulevard, Philadelphia, PA**

Novel Therapeutics and Democratization of Access: Implementation of Translational Science

Monday, October 12 (Day 1)

8:00 AM Registration and Breakfast: Smilow Lobby

8:45

Introduction

J. Larry Jameson, M.D., Ph.D., Executive Vice President, University of Pennsylvania for the Health System, and Dean, Perelman School of Medicine, University of Pennsylvania

Session 1: Models of Drug Development

Chairs: Robert H. Vonderheide, M.D., DPhil, Director, Abramson Cancer Center; Vice Dean, Cancer Programs, Perelman School of Medicine; Vice President, Cancer Programs, University of Pennsylvania Health System; University of Pennsylvania and Frances E. Jensen, M.D., FACP, Professor and Chair, Department of Neurology, Co-Director Penn Medicine Translational Neuroscience Center, Perelman School of Medicine, University of Pennsylvania

9:00

Leveraging Translational Studies to Meet the Challenges of Neuroscience Drug Discovery

Fiona Marshall, Ph.D., VP Head of Discovery Research Merck Research Laboratories.

9:30

Learning from Every Cancer Patient in Developing and Delivering Cancer Medicines

Michael Vasconcelles, M.D., Chief Medical Officer, Flatiron Health

10:00

Coffee break

Session 2: Democratization of access to the benefits of Translational Science

Chairs: Carolyn Cannuscio, Sc.D., Associate Professor of Family Medicine and Community Health, Perelman School of Medicine, University of Pennsylvania and Daniel J. Rader, M.D., Seymour Gray Professor of Molecular Medicine, Chair, Department of Genetics, Chief, Division of Translational Medicine and Human Genetics, Department of Medicine, Perelman School of Medicine, University of Pennsylvania

10:30 **Legal and Ethical Issues Relevant to Genetic Self-Exploration Tools**
Christi Guerrini, J.D, MPH, Assistant Professor, Center for Medical Ethics and Health Policy, Baylor College of Medicine

11:00 **Bringing Research from Bedside to Population: ASCVD Risk Reduction Efforts in Philadelphia**
Kevin G. Volpp, M.D., Ph.D., Founders Presidential Distinguished Professor, Perelman School of Medicine and the Wharton School; Director, Penn Center for Health Incentives and Behavioral Economics (CHIBE), University of Pennsylvania

11:30 **Balancing Innovation and Affordable Drug Prices: A Patient Perspective**
David Mitchell, Cancer Patient and Founder, Patients for Affordable Drugs

12:00 PM Lunch break

Session 3a: The Human as a Model Organism

Chairs: Mark L. Kahn, M.D., Cooper-McLure Professor of Medicine, Division of Cardiovascular Medicine, Perelman School of Medicine, University of Pennsylvania and George Cotsarelis, M.D., Milton B. Hartzell Professor and Chairman, Department of Dermatology, Director, Program on Epithelial Regeneration and Stem Cells, University of Pennsylvania Institute for Regenerative Medicine, Director, University of Pennsylvania Hair and Scalp Clinic, Perelman School of Medicine, University of Pennsylvania

1:00 **Growing Organs In Vivo: iPS Cell-Derived, Xeno-Created Organs for Transplantation**
Hiromitsu Nakauchi, M.D., Ph.D., Professor, Institute of Stem Cell Biology and Regenerative Medicine, Department of Genetics, Stanford University School of Medicine

1:30 **Human Skin Biology**
Fiona M. Watt, M.A., DPhil., Professor, King's College London

2:00 **Utilization of Single-Cell Genomics to Guide Therapeutic Decisions in Severe Drug Hypersensitivity**
Keisuke (Chris) Nagao, M.D., Ph.D., Earl Stadtman Investigator & Head, Cutaneous Leukocyte Biology Section, Dermatology Branch, National Institutes of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), National Institutes of Health

2:30 Coffee break

Session 3b: The Human as a Model Organism

Chairs: Marylyn Ritchie, Ph.D., Professor of Genetics, Associate Director for Bioinformatics, Institute for Biomedical Informatics, Perelman School of Medicine, University of Pennsylvania, and Mitchell Schnall M.D., Ph.D., Eugene P. Pendergrass Professor and Chairman of Radiology, Perelman School of Medicine, University of Pennsylvania

3:00 **Whole-body Imaging in Health and Disease**
Madeleine Cule, Ph.D., Principal Data Scientist, Calico Life Sciences

3:30 **Precision Medicine is Enhanced with Metabolomics and MRI Whole Body Imaging**
C. Thomas Caskey, M.D., FACP, FACMG, FRSC, Professor of Molecular and Human Genetics, Baylor College of Medicine

4:00 **Precision Healthcare through Multi-Scale Biomedical Data Integration**
Jessilyn Dunn, Ph.D., Assistant Professor, Biomedical Engineering and Biostatistics & Bioinformatics, Duke University

4:30 **GDF15, From Biomarker to Hormone**
Stephen O'Rahilly, MD, Professor, Institute of Metabolic Science, University of Cambridge

7:30 **Reception and Dinner: The College of Physicians of Philadelphia**
19 South 22nd Street, Philadelphia
Dinner Speaker: Edward Luce. Chief US Commentator, Financial Times. "The Mother of all Elections,"

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 13 (Day 2)

8:30 AM Registration and Breakfast: Smilow Lobby

Session 4: *The Science of Novel Therapies*

Chairs: Keith Peters, FRS, Emeritus Regius Professor of Physic, University of Cambridge and Sara Cherry, Ph.D., Professor of Pathology and Laboratory Medicine and Microbiology, Department of Microbiology, Scientific Director, High-throughput Screening Core Director, Program for Chemogenomic Discovery, Perelman School of Medicine, University of Pennsylvania

9:00 ***How Poliovirus Became a Treatment for Brain Cancer***

Annick Desjardins, M.D., FRCPC, Associate Professor of Neurosurgery and Neurology, Director of Clinical Research, The Preston Robert Tisch Brain Tumor Center at Duke, Duke University Medical Center

9:30 ***Targeted Delivery of Lipid Nanoparticles Containing Nucleoside-Modified mRNA Therapeutics***

Drew Weissman, M.D., Ph.D., Professor of Medicine, Perelman School of Medicine, University of Pennsylvania.

10:00 ***New Directions with New CAR T Cells***

Marcela V. Maus, M.D., Ph.D., Director, Cellular Immunotherapy Program, Attending Physician, Heme/Onc, Massachusetts General Hospital, Assistant Professor of Medicine, Harvard Medical School, Associate Member, Broad Institute of Harvard and MIT, Harvard Medical School

10:30 ***The Role of Oxidized Phospholipids in Inflammation: From Bench to the Clinic***

Joseph L. Witztum, M.D., Professor of Medicine, University of California, San Diego

11:00 Coffee break

Session 5: *Translational Challenges in Zoonoses*

Chairs: Susan R Weiss, Ph.D., Professor and Vice Chair, Department of Microbiology, Co-Director, Center for Research on Coronaviruses and Other Emerging Pathogens, Perelman School of Medicine, University of Pennsylvania and John H. Wolfe, VMD, Ph.D., Professor, Departments of Pathobiology and Pediatrics, School of Veterinary Medicine and Perelman School of Medicine, Director, Walter Flato Goodman Center for Comparative Medical Genetics, University of Pennsylvania and Stokes Investigator, Children's Hospital of Philadelphia

11:30 ***Development of Diagnostic, Vaccine and Therapeutic Solutions Within a Not-for-Profit Structure.***

Gary Kobinger, Ph.D., Professor of Microbiology-Infectiology and Immunology, Université Laval, Quebec City

- 12:00 PM ***Be Prepared for the Worst – A Lesson From Italy***
Giuseppe Remuzzi, M.D., Director, Mario Negri Institute for Pharmacological Research, Italy
- 12:30 ***Research in the Context of Epidemics: WHO's Perspective***
Ana Maria Henao Restrepo, M.D., MSc, Unit Lead, R&D Blueprint, WHO Health Emergencies Preparedness & Response, World Health Organization
- 1:00 Lunch break

Session 6: *Biomarkers of Disease and Drug Response*

Chairs: Jason H. Moore, Ph.D., FACMI, Edward Rose Professor of Informatics, Director, Institute for Biomedical Informatics, Director, Division of Informatics, Department of Biostatistics, Epidemiology, & Informatics, Senior Associate Dean for Informatics, The Perelman School of Medicine, University of Pennsylvania and Zoltan Arany, Ph.D., Professor of Medicine, Perelman School of Medicine, University of Pennsylvania

- 2:00 ***The Role of FGF21 in the Exocrine Pancreas Reveals a Potential Treatment for Pancreatitis***
David Mangelsdorf, Ph.D., Professor, Department of Pharmacology; Investigator, Howard Hughes Medical Institute, University of Texas Southwestern Medical Center
- 2:30 ***Beyond Protein-Coding Genes – Role of Long Non-Coding RNAs in Cancer Precision Medicine***
Stephanie Huang, Ph.D., Associate Professor and Associate Director for the Institute of Personalized Medicine, University of Minnesota
- 3:00 ***Moving the Throttle for Retinal Gene Therapy to Pandemic Speed***
Jean Bennett, M.D., Ph.D., F.M. Kirby Professor of Ophthalmology, Perelman School of Medicine, The University of Pennsylvania
- 3:30 ***Big Data and Health***
Michael Snyder, Ph.D. Professor and Chair of Genetics, Stanford School of Medicine
- 4:00 ***Concluding Remarks***
Garret A. FitzGerald, M.D., FRS, University of Pennsylvania