Harvard-MIT Regulatory Science Symposium 2018

AGENDA

8:30 AM Registration and Light Breakfast

Session 1: Overview of the Center for Regulatory Science in 2018

9:00 AM Welcome and Brief Remarks
Peter Sorger, PhD - *Harvard Medical School*

9:05 AM [Keynote] Inventing New Therapies: Where Do They Come From and How Should They Be Evaluated?
Michael Rosenblatt, MD - *Flagship Pioneering*

9:45 AM Introducing the Harvard-MIT Center for Regulatory Science (HMCRS)
Peter Sorger, PhD - *Harvard Medical School*

10:05 AM HMCRS 2018 Goals and Programs
Brian Alexander, MD, MPH - *DanaFarber Cancer Institute*

10:40 AM Morning Coffee Break

Session 2: Biomarker Development and Applications

11:05 AM Custodial Responsibilities in the Connected Age: Digital Specimens and Social Contracts
Andy Coravos - *Elektra Labs*

11:25 AM The App Store for Regulatory Science
Ken Mandl, MD, MPH - *Boston Children's Hospital*

12:00 PM Healthcare Database Analyses of Medical Products for Regulatory Decision Making
Sebastian Schneeweiss, MD, ScD - *Brigham and Women's Hospital*

12:35 PM Lunch

Session 3: Regulatory Opportunities for Improvement in the Clinical Environment

1:50 PM Tackling Acute Kidney Injury: Using High-Throughput Screening to Identify New Targets in Tubular Regeneration
Maria Beatriz Monteiro, PhD - *Harvard Medical School*

2:10 PM Pediatric Drug Regulation Reaches Adolescence
Florence Bourgeois, MD, MPH - *Boston Children's Hospital*

2:45 PM The FDA's Breakthrough Therapy Designation: Origins and Four Years of Experience
Aaron Kesselheim, MD, JD - *Brigham and Women's Hospital*

3:20 PM Afternoon Coffee Break

Session 4: Making Progress in Regulatory Science

3:55 PM The Impact of Patient-to-Patient Variability on Combination Cancer Therapy
Adam Palmer, PhD - *Harvard Medical School*

4:30 PM Advancing Biomanufacturing Regulatory Science through Collaboration
Jacqueline Wolfrum, PhD - *MIT Center for Biomedical Innovation*

5:05 PM Closing Remarks
Brian Alexander, MD, MPH - *Dana Farber Cancer Institute*

5:15 PM Drinks Reception

6:30 PM End of Symposium

SPEAKERS

Michael Rosenblatt, MD

Chief Medical Officer, Flagship Pioneering

Dr. Rosenblatt's career has been in academia, the pharmaceutical industry and biotech/venture. He is a physician, scientist, and educator. He currently serves as Chief Medical Officer of Flagship Pioneering in Cambridge, MA, a firm that creates new biotech companies. Appointed in 2016, he is the first person to serve in this role. He also was the first Executive Vice President and Chief Medical Officer for Merck (2009-2016). Previously he served as Dean of Tufts University School of Medicine. Prior to that, he held the appointments of Robert Ebert Professor of Molecular Medicine and then George R. Minot Professor of Medicine at Harvard Medical School. He served as the President of Beth Israel Deaconess Medical Center (BIDMC) from 1999-2001. Previously, he was the Harvard Faculty Dean and Senior Vice President for Academic Programs at BIDMC and served as Director of the Harvard-MIT Division of Health Sciences and Technology. The first time he was at Merck, he was Senior Vice President for Research, where he co-led the worldwide development team for alendronate (FOSAMAX), Merck's bisphosphonate for osteoporosis. He is the recipient of the Fuller Albright Award for his work on parathyroid hormone, the Vincent du Vigneaud Award in peptide chemistry and biology, and the Chairman's Award from Merck. His major research has been in parathyroid hormone--receptor interactions, elucidating the mechanisms by which breast cancer "homes" to bone, and osteoporosis. Dr. Rosenblatt has been an active participant in the biotechnology industry, serving on the board of directors and scientific advisory boards of several biotech companies. He was a scientific founder of ProScript, which discovered bortezomib (Velcade) for multiple myeloma, and Radius Pharmaceuticals, a women's health company which developed abaloparatide (Tymlos) for osteoporosis. Dr. Rosenblatt is a member of the Harvard Medical School Board of Fellows, and the research advisory committees of the Massachusetts General Hospital, the Brigham and Women's Hospital, and Boston Children's Hospital. He received his undergraduate degree summa cum laude from Columbia and his M.D. magna cum laude from Harvard. His internship, residency, and endocrinology training were all at the Massachusetts General Hospital.

Peter Sorger, PhD

Otto Krayer Professor of Systems Pharmacology, Harvard Medical School
Head, Harvard Program in Therapeutic Science
Director, Laboratory of Systems Pharmacology

Dr. Sorger received his AB from Harvard College, PhD from Trinity College, Cambridge University U.K., under the supervision of Hugh Pelham and trained as a postdoctoral fellow at the University of California, San Francisco with Harold Varmus and Andrew Murray. Prior to coming to HMS Dr. Sorger served as a Professor of Biology and Biological Engineering at MIT. His research focuses on the systems biology of signal transduction networks controlling cell proliferation and death, the dysregulation of these networks in cancer and inflammatory diseases and the mechanisms of action of therapeutic drugs targeting signaling proteins. The Sorger group also develops open-source software for analyzing biological networks and participates in multiple collaborative programs working to improve data reproducibility. As founding head of the Harvard Program in Therapeutic Sciences (HiTS), Dr. Sorger leads a university-wide effort to advance the basic and translational science used to develop new medicines, identify responsive patients and evaluate new drugs via precision clinical trials. He was co-founder of Merrimack Pharmaceuticals and Glencoe Software and is an adviser to multiple public and private companies and research institutes in the US, Europe and Japan.

Brian Alexander, MD, MPH

Associate Professor, Radiation Oncology, Harvard Medical School
Disease Center Leader, Radiation Oncology, Center for Neuro-Oncology,
Dana-Farber Cancer Institute

Dr. Alexander is an Associate Professor of Radiation Oncology at Harvard Medical School and is Disease Center Leader in Radiation Oncology for the Center for Neuro-Oncology at Dana-Farber Cancer Institute. Dr. Alexander's clinical interest is in tumors of the central nervous system. His research focuses on regulatory and translational science, particularly with regards to the generation of meaningful preclinical and early phase clinical data to support the development of novel therapies and biomarkers. He received his medical degree at the University of Michigan and completed a radiation oncology residency at the Harvard radiation oncology program. He is board certified in radiation oncology.

Ariel Stern, PhD

Assistant Professor, Harvard Business School

Dr. Stern is an Assistant Professor of Business Administration and Hellman Faculty Fellow at Harvard Business School. Her research focuses on the management of innovation in health care, with a special interest in the medical device and pharmaceutical industries. Dr. Stern is particularly interested in the intersection of the regulation, firm strategy, and economics of health care. Her research has been cited by Bloomberg, The New York Times, and National Public Radio. She received her Ph.D. from Harvard, where she was a National Bureau of Economic Research Predoctoral Fellow in the Economics of Health and Aging, and an undergraduate degree from Dartmouth College, where she was a Presidential Scholar.

Andy Coravos

CEO, Elektra Labs
Fellow at Computational Health Informatics Program (CHIP), Boston Children's Hospital

Andy Coravos is the CEO/co-founder of Elektra Labs, an open-source digital medicine project. Previously, she was a software engineer at Instacart and also Akili Interactive, a startup building clinically-validated cognitive therapeutics that look and feel like high-quality video games. Earlier in her career she worked at KKR Capstone, a private equity shop, and at McKinsey, a management consulting firm, focusing on the healthcare industry. She's a digital rights advocate and has published pieces for NeuroTechX, Huffington Post and on her blog.

Ken Mandl, MD, MPH

Director, Computational Health Informatics Program, Boston Children's Hospital
Donald A.B. Lindberg Professor of Pediatrics and Professor of Biomedical Informatics, Boston Children's Hospital

Dr. Mandl (Twitter @mandl) is Professor of Biomedical Informatics and of Pediatrics at Harvard Medical School and Director of the Boston Children's Hospital Computational Health Informatics Program. Through scholarship intersecting epidemiology and informatics, Mandl pioneered use of IT and big data for population health, discovery, patient engagement and care redesign. Dr. Mandl leads the transformative SMART Health IT initiative to design the "app store for health" and is principal investigator of the Scalable Collaborative Infrastructure for a Learning Health System across Boston hospitals and nationally. Recognized for research and teaching, Dr. Mandl received the Presidential Early Career Award for Scientists and Engineers and the Clifford A. Barger Award for top mentors at Harvard Medical School. He was advisor to two Directors of the CDC and chaired the Board of Scientific Counselors of the NIH National Library of Medicine. His clinical training and experience is in pediatrics and pediatric emergency medicine. Dr. Mandl has been elected to multiple honor societies including the American College of Medical Informatics, American Society for Clinical Investigation, Society for Pediatric Research, and American Pediatric Society.

Sebastian Schneeweiss, MD, ScD

Professor of Medicine and Epidemiology, Harvard Medical School
Vice Chief of the Division of Pharmacoepidemiology and Pharmacoeconomics, Brigham and Women's Hospital

Dr. Schneeweiss is Professor of Medicine and Epidemiology at Harvard Medical School and Vice Chief of the Division of Pharmacoepidemiology, Department of Medicine, Brigham and Women's Hospital. His research focuses on the comparative effectiveness and safety of biopharmaceuticals for regulatory decision making. He has developed analytic methods to improve the scientific validity of epidemiologic analyses using complex longitudinal healthcare databases for newly marketed medical products. The overarching theme of his research is applying advanced real-world data analytics for regulatory decision making transparently and in rapid cycles. His work is published in >400 articles. His work is funded by NIH, PCORI, Arnold Foundation, and FDA where he is also a voting consultant.

Maria Beatriz Monteiro, PhD

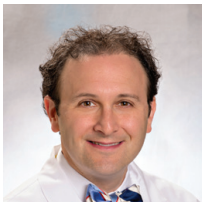
Postdoctoral Fellow, Harvard Medical School

Dr. Monteiro is a postdoctoral fellow in the Laboratory of Systems Pharmacology since September/2016. Her work has been focused on kidney disease and toxicology working on a high-throughput screen to identify novel targets for kidney tubular regeneration after acute injury and on the development of a quantitative approach to screen for new nephrotoxicants in vitro in collaboration with Pfizer, Inc. She received her doctorate degree in Sciences from University of Sao Paulo Medical School (Brazil) in January/2016. After her graduation she was awarded with the Post-doctorate Research Fellowship from the Sao Paulo Research Foundation, becoming a postdoctoral fellow in the HiTS program. Dr. Monteiro is author of 12 peer-reviewed articles (four as first author). Recently her work in kidney regeneration received 3 awards from the Society of Toxicology.

Florence Bourgeois, MD, MPH

Associate Physician in Medicine, Boston Children's Hospital
Assistant Professor of Pediatrics and Emergency Medicine, Harvard Medical School

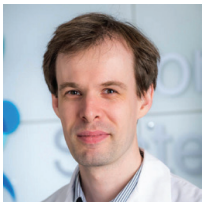
Dr. Bourgeois, MD, MPH is Assistant Professor in Pediatrics at Harvard Medical School and Director of the Initiative in Pediatric Therapeutics and Regulatory Science in the Computational Health Informatics Program at Boston Children's Hospital. Dr. Bourgeois' research interests are in the regulation and use of medications in children and the exploration of gaps in pediatric drug evidence at the point of care. She has led studies investigating the regulation of drugs and devices in pediatric populations, including evaluating the adequacy of pre-market pediatric safety and efficacy assessments, developing standardized metrics to assess the impact of FDA's regulatory programs, and defining underrepresented patient groups in pediatric drug trials. She is the recipient of an Innovation in Regulatory Science Award from the Burroughs Wellcome Fund to evaluate the epidemiology of off-label drug and biologic use in children and improve provider access to benefit-risk information on FDA-regulated products. Most recently, Dr. Bourgeois served as an Expert Visitor to the European Medicines Agency to analyze the EU's pediatric drug legislation.



Aaron Kesselheim, MD, JD

Program On Regulation, Therapeutics, And Law
Division of Pharmacoepidemiology and Pharmacoeconomics
Brigham and Women's Hospital

Aaron Kesselheim MD JD MPH is an Associate Professor of Medicine at Harvard Medical School and a faculty member in the Division of Pharmacoepidemiology and Pharmacoeconomics in the Department of Medicine at Brigham and Women's Hospital. Within the Division, Dr. Kesselheim created and leads the Program On Regulation, Therapeutics, And Law (PORTAL, www.PORTALresearch.org), an interdisciplinary research core focusing on intersections among prescription drugs and medical devices, patient health outcomes, and regulatory practices and the law. With 3 full-time faculty members, 5 post-docs, and numerous student affiliates, PORTAL is now among the largest, independent academic centers focusing on these issues in the country (Twitter: @PORTAL_research, @akesselheim).



Adam Palmer, PhD

Postdoctoral Fellow, Harvard Medical School

Dr. Palmer is a postdoctoral fellow with Peter Sorger at Harvard Medical School's Laboratory of Systems Pharmacology. Dr. Palmer investigates the mechanisms by which combination cancer therapies are clinically effective, using experimental studies of drug interactions and the evolution of drug resistance, and computational analysis of data from clinical trials and patient-derived tumor xenografts. From his discoveries of how inter-tumor and intra-tumor heterogeneity affect the clinical success of combination cancer therapies, Dr. Palmer is developing novel methods for the design and analysis of drug trials in humans and in mouse models. Previously he completed his Ph.D in Systems Biology at Harvard University with Dr. Roy Kishony, studying how mechanisms of drug action determine the evolution of antibiotic resistance.

Jacqueline Wolfrum, PhD

Associate Director, BioMAN
MIT Center for Biomedical Innovation

Dr. Wolfrum has been at the MIT Center for Biomedical Innovation since 2014. She is Associate Director of the Biomanufacturing Program (BioMAN), a pre-competitive biopharmaceutical industry consortium focused on development of new knowledge, science, technologies, and strategies that advance the manufacture and global delivery of high-quality biologic medicines. She manages sponsored projects and activities for BioMAN, focusing on initiatives in cell and gene therapy manufacturing. She has over fifteen years of experience in research, development, and manufacturing technical support for cGMP cell therapy products and cell-based medical devices in previous positions at Genzyme Biosurgery and W. R. Grace. Dr. Wolfrum received an SB in Chemical Engineering from MIT and a PhD in Biomedical Engineering from RPI.

About the Harvard-MIT Center for Regulatory Science

The Harvard-MIT Center for Regulatory Science (HMCRS) is a forum for scientists, engineers, and physicians from academic institutions, pharmaceutical and biotechnology companies, and the FDA to jointly investigate innovative strategies for effective development and evaluation of therapeutic products. By applying a systems engineering approach to drug and device safety and efficacy, the Center will accelerate the introduction of new treatments for serious diseases, determine how best to identify patients who will benefit from these treatments, and reduce the economic burden of biomedical innovation.