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# Health outcomes research in an era of cost containment

Improving efficiency of research: decreasing costs, increasing quality



**PART 1: OBSERVATIONAL STUDIES**  
**FEBRUARY 12, 2015**



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**COLUMBIA UNIVERSITY EPIDEMIOLOGY**  
SCIENTIFIC SYMPOSIUM

 **COLUMBIA UNIVERSITY** | **MAILMAN SCHOOL of PUBLIC HEALTH** **EPIDEMIOLOGY**

**PART 1: OBSERVATIONAL STUDIES  
FEBRUARY 12, 2015**

Columbia University Medical Center,  
New York City

Dear colleagues,

Welcome to today's symposium, Health outcomes research in an era of cost containment. Improving efficiency of research, decreasing costs, increasing quality. This event is cosponsored by the Centre de Recherche INSERM Epidemiologies et Biostatistique, PRES Sorbonne Paris Cité and is the first of a pair of symposia addressing health outcomes research. We will focus our attention today on observational studies.

Broadly, this is part of a symposium series hosted by Columbia University dedicated to exploring cutting edge scientific topics in epidemiology and population health (see CUESS.org). This invitation-only series brings together leaders in the field and aims to explore both the state-of-the science and to push the research agenda forward. Today's symposium is particularly exciting because it is the first such event conceived as part of an active collaboration between INSERM UMR 1153 and Columbia. Part 2, addressing interventional studies, will take place in Paris one month from today on March 12.

We are excited about this event, and hope that it will capitalize on the growing interest in innovative health outcomes research both on observational and interventional studies.

Thanks go to the faculty members here and in Paris who organized this symposium, as well as to the former Chair of Epidemiology here at Columbia, Dr. Sandro Galea, under whose leadership the series was conceived. Thanks also to the administrators whose hard work makes it all go smoothly.

Warm regards,



Neil. W. Schluger  
Professor of Medicine, Epidemiology and Environmental Health Sciences  
Interim Chair  
Department of Epidemiology

# AGENDA

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**8:30 - 9:00**    **BREAKFAST & INFORMAL GREETINGS**

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**9:00 - 9:05**    **WELCOMING REMARKS**

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**9:05 - 9:15**    **SYMPOSIUM OVERVIEW**

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**9:15 - 10:45**    **PANEL 1: FACILITATING DATA SHARING IN OBSERVATIONAL STUDIES**

**MODERATOR**    **Alfred I. Neugut, MD, PhD**

Transnational access to official micro-data: Lessons from the Data without Boundaries European project and future perspectives

**Roxane Silberman, PhD**

The MSKCC Web Survey Core and clinical research: Toward data integration

**Andrew Vickers, DPhil**

Policy on data sharing: The Whitehall experience

**Archana Singh Manoux, PhD**

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**10:45 - 11:00**    **BREAK**

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**11:00 - 12:30**    **PANEL 2: BLURRING THE LINES: EMBEDDING CLINICAL INTERVENTIONS INTO OBSERVATIONAL STUDIES**

**MODERATOR**    **Isabelle Boutron, MD, PhD**

Beyond RCTs: Exploring biopharmaceutical value and outcomes using epidemiologic approaches

**John Doyle, DrPH**

Embedding clinical interventions into observational studies: Perspectives from an NHLBI working group

**Larissa Avilés-Santa, MD, MPH, FACP, FACE**

Pragmatic trials using routinely collected electronic records

**Tjeerd-Pieter Van staa, MD, PhD**

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**12:30 - 1:15**    **LUNCH**

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**1:15 - 2:45**    **PANEL 3: CAPITALIZING ON NON-TRADITIONAL DATABASES: TAKING ADVANTAGE OF BIG DATA**

**MODERATOR**    **Mary Beth Terry, PhD**

Analysis of large-scale observational data: Opportunities and challenges

**F. DuBois Bowman, PhD**

Searching for better disease surveillance? Real-time data integration to support operational public health: The Distribute project

**Donald R. Olson, MPH**

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**2:45 - 4:15**    **PANEL 4: NEW DATA COLLECTION AND FOLLOW-UP TECHNIQUES: BRAVE NEW DIGITAL WORLD**

**MODERATOR**    **Philippe Ravaud, MD, PhD**

Evaluating performance of risk identification methods through a large-scale simulation of observational data

**Patrick B. Ryan, PhD**

Real time analysis of social media to survey drugs: The experience of Treato

**Gideon Mantel**

An example of a web-based cohort in the field of nutrition: The NutriNet-Santé study

**Serge Hercberg, MD, PhD**

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**4:15**    **CLOSING REMARKS**

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# HOSTS



**PHILIPPE RAVAUD, MD, PhD**

Adjunct Professor of Epidemiology,  
Columbia University

Professor of Epidemiology, Paris  
Descartes University (France)

Director, French Cochrane Center

Dr. Ravaud is a professor of epidemiology at Paris Descartes University and adjunct professor of epidemiology at the Mailman School of Public Health (Columbia University, New York City); director of the INSERM Epidemiology and Biostatistics Research Center, Sorbonne Paris Cité (INSERM UMR 1153); director of the Centre of Epidemiology at Hotel-Dieu; director of the French Cochrane Centre; and director of the French EQUATOR center. For more than 15 years, his primary focus has been evaluating and developing methodological research to assess treatments for patients with chronic diseases. His research activities during the last years have been structured around the following 4 main themes: evaluation of nonpharmacological treatments, development and validation of endpoints, meta-analyses and network meta-analyses, and research on research. His publication record includes more than 340 peer-reviewed papers, including more than 35 papers in the “big 6 journals” (New England Journal of Medicine, Lancet, JAMA, Annals of Internal Medicine, British Medical Journal and PLOS Medicine).



**ALFRED I. NEUGUT, MD, PhD, MPH**

Myron M. Studner Professor of  
Cancer Research (in Medicine);  
Professor of Epidemiology, Columbia  
University Medical Center

Dr. Neugut's major interests have been on GI tract cancers, notably the epidemiology of colorectal adenomas and cancer, as well as colonoscopic screening, during his time as a practicing medical oncologist and cancer epidemiologist. He serves as co-principal investigator of the Long Island Breast Cancer Study Project, a large multi-center effort to explore environmental causes of breast cancer on Long Island. Dr. Neugut focuses a great deal on racial disparities in incidence and survival from cancer and, in particular, variations across subgroups of people of African descent. He leads two large training grants in cancer-related population sciences.

# ATLANTIC ALLIANCE FOR PUBLIC HEALTH



## MOÏSE DESVAREUX, MD, PHD

Associate Professor of Epidemiology,  
Columbia University

Directeur de Recherche, INSERM  
Epidemiology and Biostatistics  
Research Center, Sorbonne Paris Cité,  
(INSERM UMR 1153)

Director, Atlantic Alliance for Public  
Health

Dr. Desvarieux's work on chronic disease built on his initial background on infectious disease. Over the last 15 years, he has been continuously funded by the US National Institutes of Health (NIH) or European agencies as PI of large national research grants with cardiovascular outcomes, particularly focusing on the interface of chronic infections, inflammation and chronic disease and relevant methods. He has also led large international consortia on the subject. His work has been published in the *Lancet*, *Circulation*, *Stroke*, *JAHA*, and other leading journals in the field. In 2005, he was awarded a Chair of Excellence from the French National Institute of Health and Medical Research (INSERM) to coordinate a consortium of collaborating cohorts in Europe, US and Asia on the subject; and received the Leadership in Research award from the Friends of the National Institute of Dental and Craniofacial Research in Washington the same year. He has fostered the collaboration between France and Columbia University's Mailman School of Public Health over the last 7 years, leading to active and expanding agreements or collaborations with EHESP, the University of Paris-SPC Center for Epidemiology and Biostatistics, INSERM and the creation of the Atlantic Alliance for Public Health, for which he received the Dean's Leadership Award in 2010.

# PARTICIPANTS



**LARISSA AVILÉS-SANTA, MD, MPH,  
FACP, FACE**

Project Director, Hispanic Community Health Study, Study of Latinos (HCHS-SOL)

Division of Cardiovascular Sciences, National Heart, Lung, and Blood Institute, NIH

Larissa Avilés-Santa, M.D. and M.P.H. joined the National Heart, Lung, and Blood Institute (NHLBI) in 2006. She is an expert on the study and prevention of diabetes and cardiovascular disease in underserved populations, particularly among Hispanic communities. Dr. Avilés-Santa is currently the Project Director for the Hispanic Community Health Study - Study of Latinos (HCHS-SOL), which was initiated on October 1, 2006. HCHS-SOL is the largest study to date to examine the prevalence of common heart disease risk factors—high blood pressure, high cholesterol, obesity, diabetes, and smoking—within a diverse Hispanic/Latino population. HCHS-SOL has enrolled over 16,000 adults of different backgrounds, including Cuban, Dominican, Mexican, Puerto Rican, Central American, and South American.



**JOHN J. DOYLE, DrPH, MPH**

Senior Vice President and Managing Director for Global Market Access & Commercialization within Advisory, Quintiles

Adjunct Assistant Professor of Epidemiology and Health Policy & Management, Columbia University

Dr. Doyle's consulting practice leads the focus on helping life sciences companies maximize the commercial success of their products through market access strategy and evidence-based marketing initiatives. Functional areas of expertise include commercial strategy, health economic and outcomes research, pricing and reimbursement, health technology assessment, and comparative effectiveness research.

Over the last two decades, Dr. Doyle has authored over 100 peer-reviewed publications in a variety of therapeutic areas, with a special concentration in oncology. Since 1993 he has worked on small-molecule chemotherapy, biologics and biosimilars including product positioning, competitive war gaming, and value-based pricing. He has lectured in the U.S., Canada, Europe, Latin America, and Asia on topics including value-based healthcare, rare disease, and health technology assessment.



**F. DUBOIS BOWMAN, PhD**

Professor and Chair, Department of Biostatistics, Columbia University

Dr. Bowman has built an active research program involving the development of biostatistical methods for brain imaging data, including functional magnetic resonance imaging, diffusion tensor imaging, and positron emission tomography. Dr. Bowman's research spans numerous substantive areas including Parkinson's disease, Alzheimer's disease, depression, schizophrenia, and cocaine addiction, among others, and has attracted substantial extramural funding.

Previously, Dr. Bowman was director of the Center for Biomedical Imaging Statistics at the Rollins School of Public Health at Emory University, a center which he founded, and tenured professor in the Department of Biostatistics and Bioinformatics. He also served as a faculty member in the Neuroscience Program in the Graduate Division of Biological and Biomedical Sciences at Emory.

Dr. Bowman is president of the Eastern North American Region (ENAR) of the International Biometric Society, and an elected Fellow of the American Statistical Association.



**SERGE HERCBERG, MD, PhD**

Full University Professor of Nutrition, University Paris 13

Hospital Practitioner, Dept Public Health, Hôpital Avicenne, Bobigny

The focus of Professor Hercberg's current work is Nutritional epidemiology and Public Health nutrition. His research pertains to major issues of public health concern, especially those with important human, social and economic consequences (cancers, cardiovascular diseases, obesity, diabetes, hypertension, cognitive decline,...). He is particularly interested 1) to study the role of nutrition (dietary intake and physical activity) as a risk or a protective factor in chronic diseases, 2) to achieve a better understanding of the determinants of dietary behaviours and nutritional status (eg., social, economic, psychological, cultural, sensory, cognitive, etc.), 3) to shed light on the underlying mechanisms (biomarkers, intermediate endpoints, etc.). His work explores also innovative methods in epidemiology such as e-epidemiology. He was PI of several large epidemiological cohorts: the SU.VI. MAX study (1994 -2007); co-PI of the SU.FOL.OM3 study (2004-2009) and is currently PI of the NutriNet-Santé study (2009-). Pr Hercberg has published more than 490 scientific journal articles and many chapters in books. He serves as member of the Haut Conseil en Santé Publique (2007-) and chair of the of the French Nutrition and Health Program, PNNS (2001-).



**GIDEON MANTEL**

Executive Chairman, Treato Pharma

With over 20 years experience in an industry built on large scale data, Gideon Mantel envisioned the impact aggregated patient generated data would have on the healthcare domain. His ability to predict market trends allowed him to turn this abstract concept into a reality years before all eyes were on digital healthcare, when he co-founded Treato. Treato analyzes billions of personal health experiences from across the social Web to create the world's most comprehensive consumer health insights platform. Treato's data serves millions of consumers, 13 of the world's largest pharmaceutical companies, leading US hospitals, as well as research and financial institutions.



**DONALD R. OLSON, MPH**

Director of the Methods Unit,  
Division of Epidemiology, New  
York City Department of Health and  
Mental Hygiene

Over the past decade Donald Olson has served as Research Scientist with the New York City Department and Mental Hygiene (DOHMH) Bureau of Communicable Diseases, visiting researcher with the Fogarty International Center NIH, Research Director for the International Society of Disease Surveillance (ISDS), and founding member and Scientific Director of the Distribute Project, a surveillance network of local, state and federal partners highlighted as a model success by the White House Office of Science and Technology Policy, and incorporated into CDC's National Syndromic Surveillance Program. Since 2012 Mr. Olson has been with the DOHMH Epidemiology Division, where he serves as Director of the Methods Unit. He received the Rick Heffernan Award for Public Health Practice in 2013, and won the Outstanding Research Article Award for Impact on the Field of Biosurveillance in 2014 for his work critically (re)evaluating the surveillance tool Google Flu Trends. Mr. Olson lives with his wife and three children in Brooklyn, New York.



**PATRICK RYAN, PhD**

Head of Epidemiology Analytics,  
Janssen Research and Development

Dr. Ryan is leading efforts at Janssen to develop and apply analysis methods to better understand the real-world effects of medical products. As part of OMOP, he is conducting methodological research to assess the appropriate use of observational health care data to identify and evaluate drug safety issues. Dr. Ryan received his undergraduate degrees in Computer Science and Operations Research at Cornell University, his Master of Engineering in Operations Research and Industrial Engineering at Cornell, and his PhD in Pharmaceutical Outcomes and Policy from University of North Carolina at Chapel Hill. Dr. Ryan has worked in various positions within the pharmaceutical industry at Pfizer and GlaxoSmithKline, and also in academia at the University of Arizona Arthritis Center.



**ROXANE SILBERMAN, PhD**

Senior Researcher, CNRS  
(Centre National de la Recherche  
Scientifique), graduate from the  
Ecole Normale Supérieure de Paris

Dr. Silberman is currently the director of Réseau Quetelet, the French Data Archives and member of Board of directors of CESSDA-AS, the European data infrastructure. As Principal Investigator for the European 7th PCRD project, Data without Boundaries (DwB), (2011-2015) with 28 partners including CESSDA Data Archives, NSIs and universities from 14 European countries, she is coordinating a project that aims at preparing the essential relationships between the European Statistical System and the Data Archives European network (CESSDA) in order to enhance transnational access to official data within the European Research Area and build a European remote access network for confidential and sensitive microdata.



**ARCHANA SINGH-MANOUX, PhD**

Research Director, Inserm

Honorary Professor, Department  
of Epidemiology & Public Health,  
University College London

Dr. Singh-Manoux currently heads the team entitled “Epidemiology of aging and age related diseases” at Inserm (France) and the cognitive ageing program of the Whitehall II study (United Kingdom). A special feature of her research has been to show the importance of heterogeneity in cognitive ageing in the transition from midlife to early old age. Over the past 10 years her group has shown robust evidence of the effects of social, behavioural, metabolic, inflammatory, and cardiovascular factors on cognitive decline. She also has a long standing research interest in social determinants of health.



**TJEERD VAN STAA, MD, PhD**

Professor of Health eResearch, Farr  
Institute of Research, University of  
Manchester

Dr. van Staa is a physician with a MSc in Pharmaco-epidemiology from McGill University. MA in Medical Ethics and PhD from Utrecht University. He is Professor of Health eResearch at the Farr Institute, University of Manchester. He has more than 20 years of significant research in pharmaco-covigilance, epidemiology, clinical trials and pharmacoecconomics. His current research activities concern pragmatic randomised trials that use routinely collected data (such as electronic health records).



**ANDREW VICKERS, DPhil**

Core Director and Attending Research  
Methodologist, Memorial Sloan  
Kettering Cancer Center

Dr. Vickers' research falls into three broad areas: randomized trials, surgical outcomes research and molecular marker studies. A particular focus of his work is the detection and initial treatment of prostate cancer. Dr Vickers has analyzed the 'learning curve' for radical prostatectomy. He is working on a series of studies demonstrating that a single measure of prostate specific antigen (PSA) taken in middle age can predict aggressive prostate cancer up to 30 years subsequently and had developed a statistical model for predicting the result of prostate biopsy based on a panel of markers. His work on randomized trials focuses on methods for integrating randomized trials into routine surgical practice so as to compare different approaches to surgery. As part of this work, he has pioneered the use of web-interfaces for obtaining quality of life data from patients recovering from radical prostatectomy and he is now core director of the Web Survey Core Facility. Dr. Vickers' methodological research centers primarily on novel methods for assessing the clinical value of predictive tools. In particular, he has developed decision-analytic tools that can be directly applied to a data set, without the need for data gathering on patient preferences or utilities.

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