



Columbia University  
Precision Medicine Initiative  
2017 Newsletter

Dear Colleagues,

Over the past year, The Columbia Precision Medicine Initiative (CPMI) has grown and taken shape with the engagement and collaboration of faculty and leadership throughout the University. With critical recruitments, a flagship lecture series, planned education programming, a major NIH award for the national 'All of Us' Precision Medicine Initiative (see below), and many other intellectual development activities, the Columbia Precision Medicine Initiative is moving forward. We are particularly pleased to welcome senior faculty Dr. Andrea Baccarelli, Dr. Philip de Jager, Dr. Charles Drake and Dr. Emmanuelle Passague.

One of the highlights of last year was our inaugural academic conference, Advances in Precision Medicine: Genetics, which saw a full day of high impact international speakers covering basic and applied science in genetics. We look forward to hosting our second conference on April 9th 2018, which will focus on cancer genomics.

Over the last year, we also had the pleasure of hosting Professors DuBois Bowman, Herbert Virgin, and Garret FitzGerald. These major figures intellectually engaged our faculty and students through the Distinguished Lecture in Precision Medicine.

I would like to take this opportunity to thank Roy Vagelos for his continuing scientific and medical leadership in precision medicine, and his generous gift to the Precision Medicine Initiative. The gift has been, and will be, used to fund a number of critical recruitments supporting precision medicine research and the infrastructure required for the necessary cutting edge science. Specifically, the Initiative has contributed start-up funds for spectacular, young researchers:

Sam Sternberg, Ph.D. Biochemistry and Molecular Biophysics; who will study the nature, evolution and application of CRISPRs to DNA editing in the Department of Biochemistry and Molecular Biology.

Alex Chavez, Ph.D. who will pursue the application of DNA editing technology to the study of human diseases in the Department of Pathology and Cell Biology.

Yueqing Peng, PhD, who uses optogenetic technology to study neural circuits involved in sleep in the Department of Pathology and Cell Biology and the Institute of Genomic Medicine.

Each of these young investigators have excelled in basic science and are committed to advancing the precision medicine effort.

I sincerely thank those of you, including scientists, engineers and clinicians from across our campuses, who are a part of this initiative, and look forward to working with you in the future. Please read on for a recap of Columbia's recent accomplishments in Precision Medicine and further details of the activities during the coming year.

Tom

# 2017 Newsletter

## Columbia University: Precision Medicine Initiative



### In this issue:

Letter from Dr. Tom Maniatis

Inaugural PMI conference

Distinguished Lectures

Faculty announcements

Medical campus updates

- *Institute for Genomic Medicine*
- *Irving Institute*
- *Discover program*
- *Precision Genomics Laboratory*

### Cross campus initiatives

- *Initiative for Structural Biology*
- *Precision Medicine and Society*
- *Pilot Grant Awards*

Educational initiatives

New website

# Advances in Precision Medicine Conference: Genetics

At the Inaugural Columbia Precision Medicine Initiative (CPMI) conference: Advances in Precision Medicine: Genetics, we heard about cutting-edge genetics research from international leaders in the field whose research advances the basic science of genetics and impacts the application of genetics to the understanding and treatment of human genetic disease.

We were honored to have these prestigious/stellar outstanding leaders in this field join us; Jonathan Pritchard, PhD, Stanford University; David Kingsley, PhD, Stanford University; Mark Daly, PhD, Harvard University; Broad Institute; Evan Eichler, PhD, University of Washington; John Hardy, PhD, University College London; Huda Zoghbi, MD, Baylor College of Medicine; Elaine Mardis, PhD, Nationwide Childrens Hospital; George Yancopoulos, MD, PhD, Regeneron Pharmaceuticals; Charles Sawyers, MD, Memorial Sloan Kettering Cancer Center; David Goldstein, PhD, Columbia University



See more from the Inaugural conference on our [website here](#).

**SAVE THE DATE: APRIL 9, 2018**

**2<sup>nd</sup> Annual Conference**

**ADVANCES IN PRECISION  
MEDICINE: CANCER GENOMICS**

**8:30am – 5:30pm**

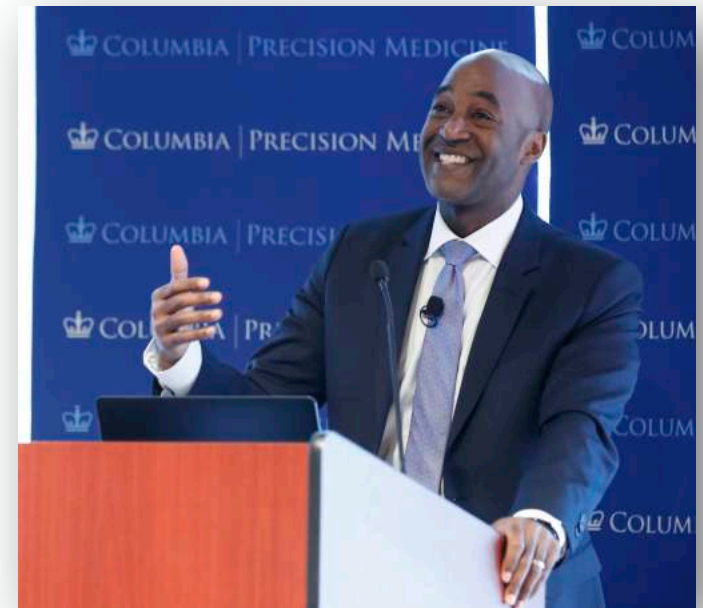
# Distinguished Lectures

## DuBOIS BOWMAN

"Statistical Methods for Linking Big Data with Precision Health"

April 18, 2017

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



## HERBERT "SKIP" VIRGIN

"Transkingdom Interactions with the Virome Regulate the Genotype/Phenotype Relationship"

March 6, 2017

Herbert W. "Skip" Virgin is the Edward Mallinckrodt Professor and Chair of the Department of Pathology & Immunology at the Washington University School of Medicine and a member of the National Academy of Sciences.

## GARRET FITZGERALD

"Precision Medicine, Translational Research, and Therapeutics"

December 5, 2016

Dr. Garret FitzGerald is the McNeil Professor in Translational Medicine and Therapeutics at the University of Pennsylvania in Philadelphia, where he chairs the Department of Systems Pharmacology.



## Upcoming 2017-2018 Distinguished Lectures:

Cori Bargmann, PhD- President, Chan Zuckerberg Science; head of the Lulu and Anthony Wang Laboratory of Neural Circuits and Behavior and the Torsten N. Wiesel Professor at the Rockefeller University

Alondra Nelson, PhD- Dean of Social Science at Columbia University; President of the Social Science Research Council

Karen E. Nelson, PhD- President of the J. Craig Venter Institute

John McHutchison, MD - Executive Vice President of Clinical Research at Gilead Sciences, Inc.

# Faculty Announcements

## DR. EMMANUELLE PASSAQUE, PhD

Emmanuelle Passegué, Ph.D., is the Alumni Professor of Genetics and Development and the Director of the Columbia Stem Cell Initiative at Columbia University Medical Center (CUMC). Her research investigates the biology of blood-forming hematopoietic stem cells in normal and deregulated contexts such as development of hematological malignancies and physiological aging.

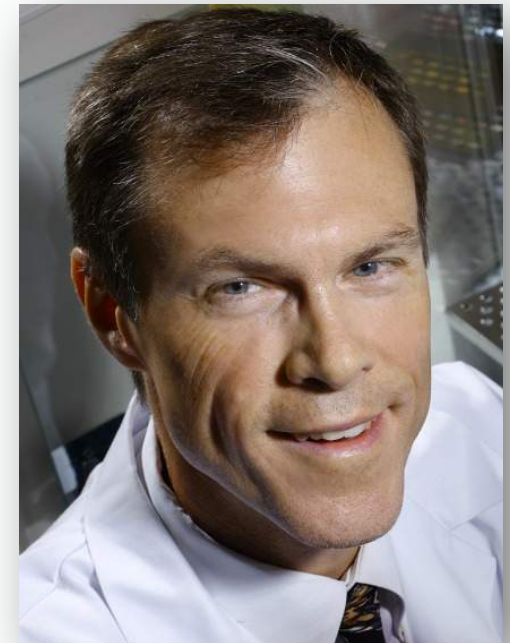


## DR. PHILIP DE JAGER, PhD, MD, MMSc

The Department of Neurology is very pleased to announce that Philip De Jager, PhD, MD, MMSc has joined our faculty as Professor of Neurology (in the Taub Institute for Research on Alzheimer's disease and the Aging Brain and the Columbia Precision Medicine Initiative).

## DR. CHARLES G. DRAKE, MD, PhD

Dr. Charles G. Drake has joined NewYork-Presbyterian/Columbia University Medical Center (CUMC) as director of genitourinary oncology and associate director for clinical research at the Herbert Irving Comprehensive Cancer Center. A nationally recognized expert in immunotherapy, Professor Drake will also serve as co-director of Columbia's Cancer Immunotherapy Programs and on the faculty of CUMC.



## DR. ANDREA BACCARELLI, MD, PhD

Andrea Baccarelli, MD, PhD serves as the Environmental Health Sciences Department Chair and the Director of the Laboratory of Precision Environmental Biosciences. As an epigeneticist and board-certified clinical endocrinologist, Dr. Baccarelli's research explores epigenetic and molecular mechanisms as potential functional pathways linking exposures to environmental pollutants to human disease. His laboratory research activities are specifically focused on epigenetics, mitochondriomics, and computational epigenomics.

# Medical Campus Updates

## Institute for Genomic Medicine



Over the past year, the IGM has made significant progress in advancing clinical practice and research for precision medicine at Columbia. The IGM's precision medicine initiatives have continued to flourish, with recruitment and enrollment of CUMC/NYP patients across a large range of specialties including epilepsy, ALS, liver and kidney disease, undiagnosed pediatric disease, ophthalmology, neuropsychiatric disease and neurodegenerative disease. These initiatives allow for the sequencing of enrolled patients, providing important diagnostic and research data for disease gene discovery and targeted treatments.

Additionally, enrollment in the national ["All of Us" Precision Medicine Initiative](#) has begun, including local community outreach and engagement activities.

Other exciting developments include the recruitment of two new IGM faculty members; [Jennifer Gelinas MD, PhD](#) and Yueqing Peng, PhD, who bring exceptional research programs in functional disease modeling in rodents, in epilepsy and sleep, respectively. Finally, the IGM has expanded its broad research portfolio to form a strategic alliance with AstraZeneca's Centre for Genomic Research. This partnership will utilize large genomic data sets, paired with cutting-edge statistical genetic and computational approaches pioneered at the IGM to uncover disease mechanisms and develop novel precision medicine based therapeutics.

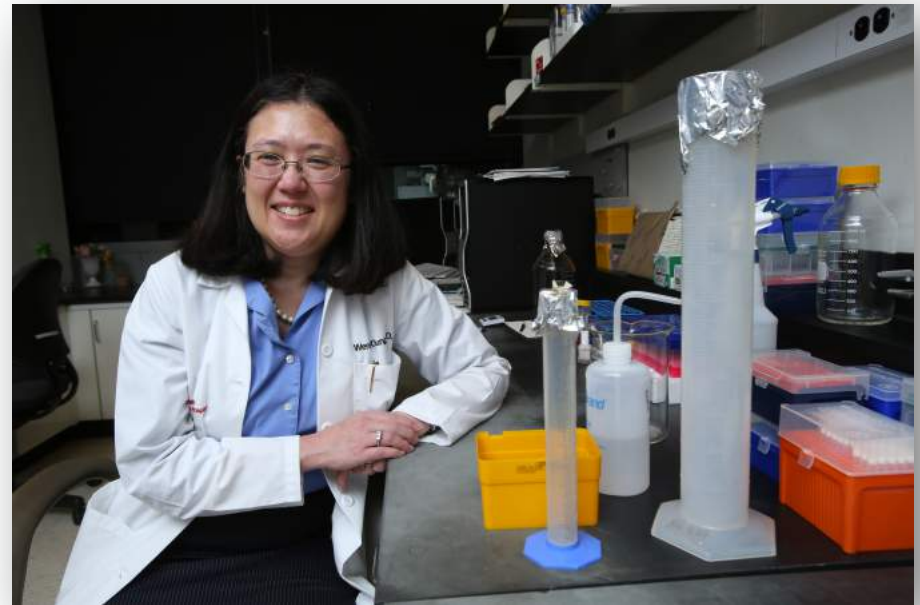
## Irving Institute for Clinical and Translational Science

In the past year, the Precision Medicine Resource team of the Institute, led by Wendy Chung, MD, PhD, and co-directors Ali Gharavi, MD, Krzysztof Kiryluk, MD, Ronald Wapner, MD, and David Goldstein, PhD, continued to develop research infrastructure, training programs, and innovative platforms to facilitate [implementation of precision medicine approaches within the Columbia community](#). The Resource supported research efforts of five new investigator teams through its pilot awards program, now in its fourth year, and welcomed two new postdoctoral/clinical fellows into its multidisciplinary training program. In January 2017 successes of the previous award recipients were celebrated at the Annual Precision Medicine Symposium that served as a platform for clinical, translational, and basic science investigators and trainees to share their cutting-edge research discoveries with a broader Columbia audience. A monthly seminar series, Advances in Precision Medicine hosted by the Resource, features thought leaders in this field from across the nation and allows physicians and scientists to engage in multi-faceted discussions on topics ranging from precision public health to challenges of genomic medicine. The Resource also launched a two-semester graduate course "Introduction to Precision Medicine" offered to health sciences students wishing to pursue a MD/MS degree and taught by the team of Columbia/Cornell faculty experts.



## DISCOVER Program

The DISCOVER program, led by Wendy Chung, MD, PhD, provides coordinated clinical services to patients with undiagnosed disorders and provides consultations and diagnostic testing for children and adults with Columbia's best diagnosticians. The program has made diagnoses in about one quarter of the patients, including genetic, infectious, and autoimmune conditions and has discovered 7 new genes for disease and connected patients to other families with the same genetic condition around the world. The DISCOVER program has supported the development of family groups for 4 genetic diseases and held family meetings to educate families about the condition and support research on these new disorders. Referrals can be made by calling 212-342-4622.



## Precision Genomics Laboratory



Recently, the Institute for Genomic Medicine (IGM) and the Department of Pathology and Cell Biology (PCB) announced the creation of the Precision Genomics Laboratory, designed to enhance constitutional genomic diagnostics, research and education at CUMC. Led by David Goldstein, PhD, Director of the IGM and Kevin Roth, MD, PhD, Chair of PCB, the PGL will offer a range of services in a CLIA-certified, CAP-accredited clinical environment and will serve the needs of patients, physicians and researchers.

Steven Spitalnik, MD will supervise the clinical laboratory and Vimla Aggarwal, MBBS, will serve as PGL medical director.

## Upcoming Events:

### RNA Saloon

October 26<sup>th</sup>, 12:00pm

Meetings are held the last Thursday of the month. *Please sign up at [tinyurl.com/RNAclub](https://tinyurl.com/RNAclub)*

New Location: Hammer 312, 701 W. 168<sup>th</sup> Street, 3<sup>rd</sup> Floor

### 2017/2018 Advances in Precision Medicine

December 14<sup>th</sup>, 4:00-5:00pm – Martin Blaser, MD, NYU

Florence Irving Auditorium, Herbert Irving Center for Cancer Research, 1st Floor, 1130 Saint Nicholas Ave. New York, NY 10032

February 8<sup>th</sup>, 4:00-5:00pm – Leslie Biesecker, MD, NHGRI

Florence Irving Auditorium, Herbert Irving Center for Cancer Research, 1st Floor, 1130 Saint Nicholas Ave. New York, NY 10032

# Cross Campus Initiatives

## Initiative for Structural Biology

An important goal of the initiative is to enable faculty research through the establishment of essential infrastructure. The growing importance of cryoelectron microscopy (CryoEM) and structure determination in precision medicine places support of this area of basic science at a very high priority. The ability to determine the importance of proteins at the atomic level is fundamentally important to advancing the frontiers of basic science, and as a tool for drug development.

We are fortunate, in this regard, to have one of the pioneers of CryoEM technology, Professor Joachim Frank, on our faculty, who we [congratulate for winning the 2017 Nobel Prize in Chemistry](#). Dr. Frank, who developed the means of determining the structures of large protein complexes such as the ribosome, established state of the art infrastructure when he joined the Columbia faculty in 2008. However, dramatic technical advances in the development of electron microscopes, high resolution cameras and computer software and Hardware over the past few years has necessitated the acquisition of expensive new equipment.



With the support of Dean Lee Goldman, and the philanthropic generosity of Lynn Shostak and Roy Vagelos, we have raised the funds necessary for a dramatic increase in the CryoEM infrastructure at CUMC and the Zuckerman Institute as well as the New York Structural Biology center. Adjunct members of the Biochemistry Department, Bridget Carragher and Clint Potter, both of whom are located at the New York Structural Biology Center, bring an innovative technology development research program in CryoEM to Columbia. In addition, there will soon be a new x-ray beam line at the Synchrotron at Brookhaven headed by Professor Wayne Hendrickson of the Biochemistry and Physiology Departments, and Dynamic structural studies have been made possible by the availability of state of the art Nuclear Magnetic Resonance (NMR) equipment, managed by Professor Art Palmer of the Department of Biochemistry at Columbia. Computational approaches to structural biology are led by Professor Barry Honig. With this extraordinary structural biology faculty, Columbia is now considered to be among the best in the world. The investment in structural biology by the CPMI also contributes to the success of the cross-university Initiative for Structural Biology, established by the Precision Medicine Initiative, and led by Professor Wayne Hendrickson, Ph.D.

This year a generous gift from Roy Vagelos has made it possible to recruit Anthony Fitzpatrick, Ph.D., currently at the Medical Research Council Laboratory of Molecular Biology in Cambridge, England, to the Department of Biochemistry. While in the U.K. Anthony applied Cryo-EM methods to the determination of the structure of Tau aggregates in samples from Alzheimer Patients, a major breakthrough in the application of CryoEM methods to the understanding of neurodegenerative diseases. As a member of the Department of Biochemistry and Molecular Biophysics, Anthony's laboratory will be located at the Zuckerman Mind Brain and Behavior Institute. He will be closely associated with the Taub Institute for research on Alzheimer's disease and the aging brain at CUMC. Anthony's multi campus engagement is an excellent example of the University-wide nature of the Precision Medicine Initiative.

## Precision Medicine and Society

Outside of the biological sciences, Columbia faculty continued pioneering the exploration of precision medicine's impacts through a diverse array of fields such as economics, law, the humanities, and sociology. With Paul Appelbaum, MD and Alondra Nelson, PhD, taking a leadership role, I am delighted that we have funded a number of projects in these fields, which you can read more about below.

Last year, Maya Sabatello, JD, PhD and Rachel Adams, PhD, developed a series of lectures and workshops on the theme of Precision Medicine: Ethics, politics and culture. You can find details of this year's lecture series [here](#).

An important partnership between Columbia University, MIT and the National Bureau of Economic Research, is on track to publish a white paper on precision medicine and economics. Columbia University, led by Jack Rowe MD, Mailman School of Public Health, hosted the NBER pre-conference last year for a two-day meeting to begin the process of commissioning scholarship. A year on and the post-meeting has wrapped; we look forward to circulating the white paper widely.

### Upcoming Events:

**Precision Medicine: Ethics, Politics, and Culture**

October 12<sup>th</sup>, 5:30-7:30pm – Dr. Kris Hult, University of Chicago

Schermerhorn Extension 754, 1200 Amsterdam Ave, New York, NY 10027

**Seminar on Ethical, Legal and Social Implications of Genetics**

November 27<sup>th</sup>, 12:00-1:00pm – Matthew Liao, PhD, Center for Bioethics & Dept. of Philosophy, NYU

Rm. 405A/B, Irving Institute for Clinical and Translational Research; 10th Fl, Presbyterian Hospital

We are delighted to announce the awardees of the inaugural Precision Medicine and Society Pilot Grants. The Precision Medicine and Society Program (part of Columbia's Precision Medicine Initiative) Pilot Grants are designed to support work on issues relating to the social, legal, economic, humanistic and ethical impact on society of the introduction of precision medicine and new genomic technologies.

These awarded projects were selected from the Precision Medicine and Society program's steering committee and reflect the breadth of interest in the social implications of precision medicine, more broadly, at Columbia.

*Comparing Precision Medicines Boundaries and Identities in China and the United States*

**Larry Au (Sociology) and Dr. Gil Eyal (Sociology)**

Compare the emergence of precision medicine in the United States and China using interviews and ethnographic observation of scientists and doctors in the two countries.

*Comparing Inclusion/Exclusion in Precision Medicine Trials and Clinical Trials*

**Adam Obeng (Sociology), Moran Levy (Sociology), and Dr. Gil Eyal (Sociology)**

An examination of how the rise of precision medicine affects inclusion of patients from diverse social groups in cancer clinical trials.

*Establishing meaningful informed consent for whole-genome/exome sequencing in pediatric oncology: A web-based educational intervention*

**Dr. Jennifer Oberg (CUMC-Pediatrics), Dr. Jennifer Levine (CUMC-Pediatrics), and Dr. Julia Bender (CUMC-Pediatrics)**

Pilot test a web-based educational intervention to facilitate informed consent for whole genome/exome sequencing in pediatric oncology and improve parent understanding of the risks and benefits of genomic testing.

*Intervening in Digital Grief with Artificial Intelligence*

**Dr. Desmond Upton Patton (School of Social Work)**

A social media-based intervention development study that will identify and analyze the sequence of traumatic and threatening content on Twitter that inform the adaptation of an emotionally intelligent chatbot that disseminates resources and supports to youth of color who express grieving and aggressive related content on Twitter.

*What Do Teenagers Think about Precision Psychiatry?*

**Dr. Maya Sabatello (CUMC-Psychiatry), Dr. Jehannine Austin (Medical Genetics, University of British Columbia), and Dr. Paul Appelbaum (CUMC-Psychiatry)**

A study of teenagers' perspectives about precision medicine in psychiatry.

**SAVE THE DATE: APRIL 20, 2018**

Genetics of the Evolutionary Process

8:30am - 5:30pm

**Location: MMBI Auditorium,  
Jerome L. Greene Science Center**

The symposium will feature talks on current evolutionary research, focused on areas of interest to the broader biology community. For questions, please contact Ellie Siddens ([mes2314@columbia.edu](mailto:mes2314@columbia.edu)). More details [here](#).

# Educational Initiatives

A key goal of Columbia's Precision Medicine Initiative is to establish new educational programs in Precision Medicine at all levels to disseminate knowledge, and to train qualified practitioners of Precision Medicine. To that end, a Genetic Counseling Masters program has been established, led by Medical Directors Ronald Wapner, MD and Wendy Chung, MD, PhD. The directors have recently appointed [Amanda Bergner, MS](#), as Program Director of the Masters, enrollment is expected to begin in Fall 2018. Central objectives of this program are to provide training in the genetics and genomics necessary for genetic counseling in precision medicine, to address the impending shortage of genetic counselors as the practice of precision medicine grows.

We continue establishing exposure to the fundamentals of precision medicine for medical students, incorporating exposure to top scientists in relevant fields, as well as at the undergraduate and graduate levels in multiple disciplines. We are also particularly pleased to be working closely with the Irving Institute for Clinical and Translational Science, led by Muredach Reilly, MD, PhD, in collaborating and promoting the Precision Medicine monthly lecture series and other activities.

Last winter, CPMI hosted the inaugural Life Science Entrepreneurship seminar series, developed by CBS alumni Lorraine Marchand, MBA and Tig Conger, MBA. The series consisted of 4 classes, each centered around a case study exploring best practices among industry leaders. Our objective was to expose graduate students and young faculty from the life science disciplines to the entrepreneurial world. Participants were able to learn from the experiences of biotechnology luminaries including George Yancopoulos, MD, PhD, Leonard Schleifer MD PhD, Roy Vagelos, MD, and Tony Evnin, PhD. We are planning a similar series again in Spring 2018.

Finally, CPMI is collaborating with the American College of Physicians to develop a series of precision medicine guidelines for physicians, that will be disseminated in ACP's Annals of Medicine. Mailman's Jack Rowe, MD is leading the Columbia effort.

New research is constantly reflected in Columbia's faculty-led education programs. The Columbia Precision Medicine Initiative will continue that trend, and expand offerings to comprehensively train academics and professionals needed for delivery of precision medicine.

Visit our new  
Precision Medicine  
website!