Center for Biologics Evaluation Research

SCIENCE SYMPOSIUM

2018

June 25-26, 2018

Food and Drug Administration

White Oak Campus

Silver Spring, Maryland

PROGRAM BOOK

WELCOME

Dear CBER Colleagues,

Welcome to the 2018 CBER Science Symposium! Conducting research related to the products that we regulate is an integral part of what we do at the Center to support public health. This symposium provides us with the opportunity for scientific exchange and relevant dialogue across the spectrum of the products regulated by the Center. The exchange over the next two days may lead to new insights or approaches regarding existing projects, or perhaps lead to novel ideas for future research. It is our hope that through such excellent communication and collaboration, CBER can ultimately maximize the contribution of its work toward the improvement of public health. Thank you for participating. We hope that you will find the program both informative and enjoyable.

Sincerely,
Peter Marks, MD, PhD, Director, CBER

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Dear CBER Colleagues,

Welcome to the 2018 CBER Science Symposium! Thank you for choosing to spend time with us over the next two days to learn, share, and discuss the latest scientific advances within and external to CBER that support our regulatory mission.

We are excited about our revised format allowing us to invite more external speakers to enrich the scientific sessions. We hope that the combination of invited external and internal speakers will allow staff to obtain updated knowledge and information about scientific research in a number of exciting areas relevant to CBER. We used a combination of polling the CBER community along with input and direction from the organizing committee to choose the topics and speakers for each of the 6 sessions.

No scientific meeting is successful without a dedicated group to plan and coordinate logistics*. I am grateful to the engaged and interactive group from OBE, OBRR, OCBQ, OCOD, OD, OTAT and OVRR who served on this year's Planning and Execution Committees. In particular, I want to thank Dr. Monica Young for her leadership in working across the Center to organize and implement all aspects of the 2018 CBER Science Symposium.

Enjoy, learn, communicate, and collaborate!

Sincerely,
Carolyn Wilson, PhD, Associate Director for Research, CBER

* see pages 4-6 for full listing

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Aikaterini Alexaki, PhD, Staff Fellow

Adnan Jaigirdar, MD, FACS, Medical Officer

Chava Kimchi-Sarfaty, MSc, PhD, Acting Deputy Associate Director for Research

Ramavati Pal, PhD, Visiting Associate

Office Vaccines Research and Review, CBER

Hana Golding, PhD, Chief, Laboratory of Retroviruses

Ramachandran Girish, PhD, Regulatory Reviewer

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FDA Fellows Association (FFA)

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Geoffrey Heinzl, PhD, FFA Co-chair & ORISE Fellow, CDER

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Paul Richards, Public Affair Specialist

Center for Biologics Evaluation Research

SCIENCE SYMPOSIUM

2018

AGENDA

Monday – June 25, 2018

9:00 AM **Opening Remarks**
RADM Denise Hinton
Chief Scientist, FDA

Session 1: Emerging and Re-Emerging Infectious Diseases

Chair: Hana Golding, PhD, Co-Chair: Hong Yang, PhD

9:15 AM **Pandemic Preparedness: Challenges and Opportunities**
Anthony Fauci, MD
Director, National Institute of Allergy and Infectious Diseases, NIH

9:45 AM **Blood Safety and Availability: Regulatory and Scientific challenges for Emerging and Re-emerging Infectious Diseases**
John Hobson, PhD
Deputy Director, Office of Blood Research & Review
Center for Biologics Evaluation & Research, FDA

10:00 AM **Benefit–Risk Assessment to Support Management of Transfusion-Transmission Risk of Infectious Diseases**
Hong Yang, PhD
Principal Investigator, Office of Biostatistics and Epidemiology
Center for Biologics Evaluation & Research, FDA

10:15 AM **The Host Response to Pertussis Vaccination and Infection**
Tod Merkel, PhD
Principal Investigator, Office of Vaccines Research & Review
Center for Biologics Evaluation & Research, FDA

10:30 AM **Break**

Session 2: Harnessing diverse types of data in regulatory decision-making

Chair: Shiowjen Lee, PhD, Co-chair: Mark Walderhaug, PhD

10:45 AM **Honest Learning for the Healthcare System: Large-Scale Evidence from Real-World Data**
David Madigan, PhD
Executive Vice President and Dean of Faculty of Arts and Sciences, Professor of Statistics
Columbia University

11:15 AM **Using Non-Experimental Evidence in Drug and Biologics Approvals**
Steven Goodman, MD, MHS, PhD
Associate Dean & Professor
Stanford University

11:45 AM **A Roadmap for Supporting the Decision-Making Process in Post-Market Safety Surveillance**
Taxiarchis Botsis, MSc, PhD
Assistant Professor of Oncology
Johns Hopkins University

12:00 PM **FDA's Monitoring of Guillain Barré Syndrome Following Influenza Vaccines Using CMS Data**
Richard Forshee, PhD
Associate Director for Research, Office of Biostatistics and Epidemiology
Center for Biologics Evaluation & Research, FDA

12:15 PM **Lunch**

Session 3: Immunogenicity and Immunotherapy

Chair: Ira Berkower, MD, Co-chair: Aikaterini Alexaki, PhD

1:30 PM **Do Differences Make a Difference: Human Immune Responsiveness and Single-Cell Variations**
John Tsang, PhD
Chief, Systems Genomics and Bioinformatics Unit, Laboratory of Immune System Biology
Co-Director, NIH Center for Human Immunology (CHI),
National Institute of Allergy and Infectious Diseases, NIH

2:00 PM **Pre-existing Immunity Shapes Neutralizing Antibody Responses to Influenza Vaccines**
Carol Weiss, MD, PhD
Laboratory Chief & Principal Investigator, Office of Vaccines Research & Review
Center for Biologics Evaluation & Research, FDA

2:15 PM **Predicting Immune Responses to Therapeutic Proteins: The Promise of Safer Drugs and Improved Clinical Outcomes**
Zuben Sauna, PhD
Principal Investigator, Office of Tissues and Advanced Therapies
Center for Biologics Evaluation & Research, FDA

2:45 PM **Development of Safe and Effective Cancer Vaccines and Cellular Immunotherapy Products**
Raj Puri, MD, PhD
Division Director & Principal Investigator, Office of Tissues and Advanced Therapies
Center for Biologics Evaluation & Research, FDA

Tuesday– June 26, 2018

9:00 AM **Opening Remarks & Recognition of CARE Abstract Competition**
Carolyn Wilson, PhD
Associate Director for Research, Center for Biologics Evaluation & Research, FDA

Session 4: New approaches to improve biologics and treatments
Chair: Monique Gelderman-Fuhrmann, PhD, Co-chair: Wojciech Jankowski, PhD

9:30 AM **The Human Cell Atlas**
Aviv Regev, PhD
Professor of Biology, Massachusetts Institute of Technology, Chair of the Faculty and Core Member, Broad Institute of MIT and Harvard
Director, Klarman Cell Observatory and Cell Circuits Program, Broad Institute
Investigator, Howard Hughes Medical Institute
Founding co-chair, Human Cell Atlas

10:00 AM **Advances in Biomedicine are Bearing Fruit: Innovative New Therapies Enter the Clinic and Biotechnology Pipeline**
Chava Kimchi-Sarfaty, PhD
Acting Deputy Associate Director for Research, Office of Tissues and Advanced Therapies
Center for Biologics Evaluation & Research, FDA

10:15 AM **High-throughput Sequencing for Adventitious Virus Detection to Enhance Safety of Biologics**
Arifa Khan, PhD
Principal Investigator, Office of Vaccines Research & Review
Center for Biologics Evaluation & Research, FDA

10:30 AM **New Approaches to Improve Stored Cellular Blood Components**
CD Atreya, PhD
Associate Director for Research & Principal Investigator
Office of Blood Research & Review
Center for Biologics Evaluation & Research, FDA

Historical Perceptive
Chair: Carolyn Wilson, PhD

10:45 AM **Immune Globulin Intravenous and Hepatitis C Virus Transmission: The Critical Role of CBER's Research**
Lawrence Bachorik, PhD
Former FDA Associate Commissioner for Communications

11:15 AM **Break**

Session 5: Microbiome in Human Disease

Chair: Siobhan Cowley, PhD, Co-chair: Sheila Dreher-Lesnick, PhD

11:30 AM **The Human Microbiome and the Relationship with Health and Disease**
Vincent Young, MD, PhD
Professor, Department of Internal Medicine/Infectious Diseases Division
Department of Microbiology and Immunology
University of Michigan

12:00 PM **Interactions Between the Immune System, the Microbiome, and *Clostridium difficile***
Paul Carlson, PhD
Principal Investigator, Office of Vaccines Research & Review
Center for Biologics Evaluation & Research, FDA

12:45 PM **Bioinformatics of Microbiome: Challenges and Solutions at FDA**
Vahan Simonyan, PhD
Lead Scientist & Project Director, High-Performance Integrated Virtual Environment
Center for Biologics Evaluation & Research, FDA

1:00 PM **Lunch**

Session 6: Advances in Regenerative Medicine

Chair: Steve Bauer, PhD; Co-chair: Ramavati Pal, PhD

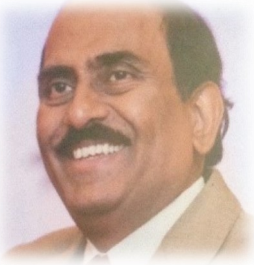
2:00 PM **3D Printing Technologies for Tissue Engineering**
Jordan S. Miller, PhD
Assistant Professor, Department of Bioengineering
Rice University

2:30 PM **Developing Strategies to Improve Characterization of Cell-based Regenerative Medicine Products**
Steven Bauer, PhD
Laboratory Chief & Principal Investigator, Office of Tissues and Advanced Therapies
Center for Biologics Evaluation & Research, FDA

2:45 PM **Combining Next Generation and Classical Methods to Reverse Engineer Neural Induction**
Malcolm Moos, MD, PhD
Principal Investigator, Office of Tissues and Advanced Therapies
Center for Biologics Evaluation & Research, FDA

3:00 PM **Practical Microscale Technologies in the Assessment of Regenerative Medicine Advanced Therapeutic Products**
Kyung Sung, PhD
Principal Investigator, Office of Tissues and Advanced Therapies
Center for Biologics Evaluation & Research, FDA

SPEAKER BIOGRAPHIES



C.D. Atreya, PhD

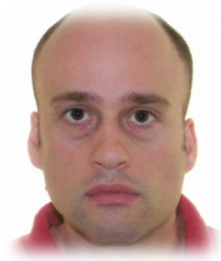
Dr. Atreya is the Associate Director for Research for the Office of Blood Research and Review at the Center for Biologics Evaluation and Research (CBER), US Food and Drug Administration, USA. He has been with FDA for over 25 years with research and regulatory expertise. Dr. Atreya's regulatory review expertise includes pediatric viral vaccines and research expertise includes virology, microbiology, cell biology. His current research activities are geared towards development of novel pathogen reduction concepts and product safety biomarkers for stored blood cells (platelets and RBC). Dr. Atreya has authored over 75 peer-reviewed scientific publications, served and/or on the editorial board for several peer-reviewed journals and wrote several book chapters.

Lawrence Bachorik, PhD

During his 33 years at the FDA (with a five-year hiatus in the 1980s as director of public affairs for a major regional health care system), Dr. Bachorik served in a variety of senior positions. He directed the FDA's media relations, communications, and external relations operations, and he also wrote speeches for every FDA Commissioner from 1979 through 2015. Since his retirement in 2015, he has taught a course on the FDA, regulation, science, and health policy at the graduate school at the National Institutes of Health. Before joining the FDA, he taught English literature at Georgetown University, George Washington University, George Mason University, and the University of Vermont. He holds a Ph.D. in English literature from McGill University.

Steven R. Bauer, PhD

Dr. Bauer is the Chief of the Cellular and Tissue Therapy Branch (CTTB), Division of Cellular and Gene Therapies (DCGT) in the Office of Tissues and Advance Therapies (OTAT) at the Center for Biologics Evaluation and Research (CBER), U.S. Food and Drug Administration (FDA). As the Chief of CTTB, Dr. Bauer supervises CBER scientific staff engaged in review of cell-based biological therapies, policy development in emerging areas of cellular therapies, and research relevant to their use in clinical trials. His current research focuses on mesenchymal stem cell biology and stromal cell-hematopoietic cell interactions that influence development of lymphocytes. Dr. Bauer received his Ph.D. in Biochemistry from the University of Maryland in 1986. From 1986 through 1991, Dr. Bauer was a scientific member of the Basel Institute for Immunology in Basel, Switzerland. In 1991, Dr. Bauer joined CBER's Division of Cellular and Gene Therapies.



Taxiarchis Botsis, PhD

Dr. Botsis is an Assistant Professor of Oncology and Medical Informatics at The Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins University School of Medicine. His background lies in the field of Medical Informatics with particular focus on the natural language processing of clinical texts and the automated coding and structuring of free-text information. One of his main interests is the development of efficient information visualizations. Dr. Botsis previously worked at the US Food and Drug Administration where he led the development of a Decision Support Environment to support medical experts in the review of post-market reports.

Paul Carlson, PhD

Paul Carlson is a principal investigator in the Laboratory of Mucosal Pathogens and Cellular Immunology in the Division of Bacterial, Parasitic, and Allergenic Products, Office of Vaccines Research and Review. He received his Ph.D. from the University of Pittsburgh and performed postdoctoral research at the University of Michigan. Since starting his laboratory at FDA in 2015, Dr. Carlson's research has focused on infections caused by the enteric pathogens *Clostridium difficile* and Vancomycin resistant *Enterococcus* species. Dr. Carlson is co-chair of the FDA microbiome working group and the Joint Agency Microbiome (JAM) working group, as well as a member of the Microbiome Interagency Working Group (MIWG). His regulatory responsibilities include product (CMC) review for fecal microbiota transplantation (FMT), defined live biotherapeutic products, and bacteriophage therapies.

Anthony S. Fauci, MD

Dr. Fauci is a physician-scientist who directs the National Institute of Allergy and Infectious Diseases (NIAID) at the National Institutes of Health in Bethesda, Maryland. He oversees an extensive research program on infectious diseases such as HIV/AIDS, influenza, tuberculosis, Ebola and Zika, as well as diseases of the immune system. Dr. Fauci also serves as one of the key advisors to the White House and Department of Health and Human Services on global infectious disease issues. He was one of the principal architects of the President's Emergency Plan for AIDS Relief (PEPFAR), a program that has saved millions of lives throughout the developing world. Dr. Fauci also is the long-time chief of the NIAID Laboratory of Immunoregulation where he has made numerous important discoveries related to HIV/AIDS and is one of the most-cited scientists in the field. He is a member of the US National Academy of Sciences and the US National Academy of Medicine and has received numerous prestigious awards for his scientific and global health accomplishments, including the National Medal of Science, the Robert Koch Medal, the Mary Woodard Lasker Award for Public Service, and the Presidential Medal of Freedom. He has been awarded 45 honorary doctoral degrees and is the author, coauthor, or editor of more than 1,300 scientific publications, including several major textbooks.

Richard A. Forshee, PhD

Dr. Forshee leads the Analytics and Benefit-Risk Assessment Team for the Office of Biostatistics and Epidemiology in the Center for Biologics Evaluation and Research at the U.S. Food and Drug Administration. He works on a wide range of issues related to the risks and benefits of blood and blood products, vaccines, and human cell and tissue products. Before joining the FDA, he was the Director of the Center for Food, Nutrition, and Agriculture Policy at the University of Maryland, College Park.

Steven Goodman, MD, MHS, PhD

Dr. Goodman is Associate Dean for Clinical and Translational Research, Professor of Medicine and of Epidemiology, and Chief of the Division of Epidemiology at the Stanford University School of Medicine. He directs the Stanford CTSA TL1 and KL2 research training programs, and is the co-founder and co-director of METRICS (Meta-Research Innovation Center at Stanford), a center dedicated to scientifically studying and improving the validity of published biomedical research. He is a senior statistical editor of Annals of Internal Medicine, serves as Vice-Chair of the Methodology Committee of the Patient-Centered Outcomes Research Institute (PCORI) and is Scientific Advisor to the Medical Advisory Panel of the national Blue Cross-Blue Shield technology assessment program. He was the editor of Clinical Trials: The Journal of the Society for Clinical Trials from 2004-2013. Before joining Stanford in 2011, for two decades he was a member of and then director of the Division of Biostatistics and Bioinformatics in the Johns Hopkins Kimmel Cancer Center. He has long written about inferential and Bayesian approaches in clinical research, and co-organized the 2004 FDA conference "Can Bayesian Approaches to Studying New Treatments Improve Regulatory Decision Making?" He was awarded the 2016 Spinoza Chair from the University of Amsterdam for his work in inference.

John Peyton Hobson, PhD

Dr. Hobson is the Deputy Director of the Division of Emerging and Transfusion Transmitted Diseases (DETTD) in the Office of Blood Research and Review at the Center for Biologics Evaluation and Research (CBER), US Food and Drug Administration. He has been with FDA for almost 10 years. Dr. Hobson received a B.S. in Biology from Salisbury State University, a M.S. in Biotechnology from Georgetown University, and a Ph.D. in Biochemistry from Georgetown University. He completed post-doctoral training at the National Institutes of Health where he worked on developing mouse models to investigate the function of cell-surface proteases. After leaving NIH he worked in the biotech industry, serving as a senior scientist responsible for developing point-of-care, multiplexed, immuno and molecular assays for a variety of host biomarkers and pathogen targets. Prior to joining DETTD, Dr. Hobson served as a Regulatory Scientist and Branch Chief in the Division of Microbiology Devices in the Office of In vitro Diagnostics and Radiological Health. Dr. Hobson is a recognized expert in the development, validation and regulation of diagnostic and donor screening devices.

Arifa S. Khan, PhD

Dr. Khan is a Principal Investigator and Supervisory Microbiologist in the Division of Viral Products, Office of Vaccines Research and Review in the Center for Biologics Evaluation and Research, U.S. Food and Drug Administration. She is also an Adjunct Professor at Georgetown University Medical Center. She joined the FDA in 1991, after working at NIAID, NIH since 1979, where she did seminal research on pathogenesis of murine retroviruses, SIV, and endogenous retroviruses. With this expertise, Dr. Khan established a research program in CBER to develop sensitive assays for detection and characterization of endogenous retroviruses and adventitious viruses for cell substrate and vaccine safety. Her current focus is evaluation of next generation sequencing technologies for virus detection and development of relevant reference materials needed for standardizing the methods for adventitious virus testing in biologics. Her regulatory responsibilities include review of Investigational New Drugs and Biological License Applications for a variety of candidate vaccines against viral diseases, including HIV and influenza, and providing consult across different product categories on adventitious virus testing for novel cell substrates. Dr. Khan has been involved in licensure of several viral vaccines and development of various FDA, ICH, PHS, and WHO guidance documents related to cell substrates, vaccines, therapeutics, and xenotransplantation. She is a leader in internal and external efforts on advanced virus detection technologies and member of several working groups including the FDA genomics working groups. Dr. Khan received her Ph.D. in Microbiology from the George Washington University, Washington, D.C.

Chava Kimchi-Sarfaty, MSc, PhD

Dr. Kimchi-Sarfaty is presently OTAT Acting Deputy Associate Director for Research and leading a group at the FDA, Division of Plasma Protein Therapeutics, Office of Tissues and Advanced Therapies (OTAT) that investigates the function of various blood coagulation factors and their relation to diseases. She was one of the first groups to demonstrate that a synonymous single nucleotide polymorphism in a *MDR*1 haplotype affects the function and conformation of P-glycoprotein folding and hence its function. Since then, synonymous mutations have assumed a focal point in her genetic research. Increased scientific awareness of the potential deleterious effects of synonymous mutations has led to the reevaluation of many recombinant protein therapeutics, which often contain one or more synonymous mutations. Her group has characterized many naturally occurring synonymous mutations with pathogenic implications using both novel and existing protein characterization techniques. Along with a strong familiarity with coagulation factors, she reviews and chairs pre-INDs, INDs and BLAs for recombinant proteins and plasma derivatives products such as von Willebrand factor, ADAMTS13, factor VIII, factor IX, thrombin and fibrinogen.

David Madigan, PhD

Dr. Madigan is Professor of Statistics and Executive Vice President and Dean of the Faculty of Arts & Sciences at Columbia University in New York City. He received a bachelor's degree in Mathematical Sciences and a Ph.D. in Statistics, both from Trinity College Dublin. He has previously worked for AT&T Inc., Soliloquy Inc., the University of Washington, Rutgers University, and SkillSoft, Inc. He has over 180 publications in such areas as Bayesian statistics, text mining, Monte Carlo methods, pharmacovigilance and probabilistic graphical models. He is an elected Fellow of the American Statistical Association, the Institute of Mathematical Statistics, and the American Association for the Advancement of Science. He has served terms as Editor-in-Chief of Statistical Science and of Statistical Analysis and Data Mining – the ASA Data Science Journal.

Tod Merkel, PhD

Dr. Merkel received his Ph.D. from the University of Virginia and received postdoctoral training at the National Institutes of Health before establishing a research program in the Food and Drug Administration. He brings over thirty years of experience in infectious disease research and related disciplines and 18 years of vaccine regulatory experience to the study of bacterial pathogenesis and the evaluation of new vaccines. He has extensive experience developing and utilizing animal models of bacterial infections, including murine and non-human primate models of Pertussis, murine models of Staphylococcal infection, and murine models of Anthrax. The baboon model of pertussis, developed in Dr. Merkel's laboratory has provided important insights into the host response to both pertussis vaccination and infection. This model continues to provide the pertussis research community with the opportunity to address important questions that shed light on the pathogenesis and vaccine-mediated protection of this important pathogen.

Jordan S. Miller, PhD

Dr. Miller received his bachelor's degree in Biology from MIT in 2003, and PhD in Bioengineering from Rice University in 2008. His primary research interests combine synthetic chemistry, 3D printing, microfabrication, and molecular imaging to direct cultured human cells to form more complex organizations of living vessels and tissues for research in regenerative medicine. Precisely engineered *in vitro* systems at the molecular, micro- and meso-scale are well suited to decouple the relationship between tissue architecture and cell function. These systems are now permitting comprehensive closed-loop design and optimization of large-scale engineered tissues through refinement with computer models of mass transport and assessment of their therapeutic potential *in vivo*.

Malcolm Moos Jr, MD, PhD

Dr. Moos majored in Biological Sciences at Stanford University and obtained his M.D. and Ph.D. at the University of Minnesota. His graduate major was pharmacology, with a supporting program in biophysics and spectroscopy. He completed residency training in Laboratory Medicine and Pathology, with a fellowship in Clinical Chemistry, at the same institution. Dr. Moos then came to CBER to continue his work in the area of cyclic nucleotide-mediated signal transduction, and became a recognized expert in protein microsequencing, separation techniques, and protein analytical biochemistry. When he began independent investigations as a Medical Officer, he changed fields to support regulatory decisions in the emerging area of cellular therapy. His current research interests are to define how evaluating the status of major cell signaling pathways (BMP, Wnt, etc.) can be used in conjunction with recent developments in systems biology, single cell analytical technology, and computational biology to characterize cell-based products more accurately to facilitate improved product design and testing. His work has resulted in seven patents issued, one allowed, and two pending. While at CBER, he has been CMC reviewer on hundreds of IND submissions (Original Submissions plus Amendments) and six BLAs, been primary or contributing author on various FDA and International Conference on Harmonisation guidance documents dealing with recombinant protein and cellular products, organized various meetings dealing with cell and gene therapy regulatory issues, and participated in criminal investigations and prosecutions when needed. His awards include the Harvey W. Wiley Medal, the FDA Award of Merit, the Center Director's Targeted Research Award (twice), the Center Director's Distinguished Service Award, and thirty-two others.

Raj K. Puri, MD, PhD

Dr. Puri is the Director of the Division of Cellular and Gene Therapies (DCGT) in Office of Tissues and Advanced Therapies (OTAT) at FDA's Center for Biologics Evaluation and Research (CBER). He is also a Chief of Tumor Vaccines and Biotechnology Branch. Prior to joining FDA/CBER, Dr. Puri was trained at National Cancer Institute's Surgery Branch in immunotherapy approaches for cancer and at Mayo Clinic, Rochester, Minnesota on hormone receptors. At FDA, Dr. Puri oversees evaluation and regulation of cancer vaccines, immunotherapy, cellular and gene therapy, tissue engineering, and xenotransplantation products and development of policies and guidance documents in these 21st Century cutting edge areas of medical research. In addition, Dr. Puri oversees and manages mission critical research performed by 11 principal investigators in DCGT to support cutting edge medical product development. Dr. Puri also directs translational research program in the field of cancer vaccines, cancer targeting and immunotherapy of cancer. Dr. Puri has done seminal research in targeting glioblastoma multiforme, a deadliest form of human brain cancer. He has discovered two receptor targeted agents (IL-4-PE and IL-13-PE) for cancer therapy and both agents are in various stages of clinical trials. This large body of work (>300 articles) has been published in numerous scientific and medical journals. In addition, Dr. Puri has been involved in the application of genomics technology in medical product development, policy and guidance documents development, outreach efforts and conducting research focusing on cancer stem cells. Dr. Puri is an Associate Editor of Immunotherapy journal, member of the editorial board of three international medical journals.

Aviv Regev, PhD

Dr. Regev, a computational and systems biologist, joined the Broad Institute as a core member and MIT as a faculty member in 2006. Regev's research centers on understanding how complex molecular circuits function in cells and between cells in tissues.Regev is a professor in the Department of Biology at MIT, Chair of the Faculty and founding director of the Klarman Cell Observatory and Cell Circuits Program at the Broad, and an Investigator at the Howard Hughes Medical Institute. Her lab has been a pioneer of single-cell genomics – inventing key experimental methods and computational algorithms in the field, and demonstrating how to apply it to understand cell taxonomies, histological organization, differentiation and physiological processes, and how to infer the

molecular and cellular circuits that control the function of cells and tissues in health and disease. She co-founded and co-leads the international initiative to build a Human Cell Atlas (HCA), whose mission is to create comprehensive reference maps of all human cells—the fundamental units of life—as a basis for both understanding human health and diagnosing, monitoring, and treating disease. Regev is a recipient of the 2008 NIH Director's Pioneer Award, the 2008 Overton Prize and 2017 Innovator Prizes from the International Society for Computational Biology (ISCB) and is a Class of 2016 ISCB Fellow, the 2014 Earl and Thressa Stadtman Scholar Award from the American Society for Biochemistry and Molecular Biology, and the 2017 Paul Marks Prize. Prior to joining the Broad Institute, Regev was a Bauer Fellow at Harvard University, where she developed new approaches to the reconstruction of regulatory networks and modules from genomic data. Regev received her M.Sc. from Tel Aviv University, studying biology, computer science, and mathematics in the Interdisciplinary Program for the Fostering of Excellence. She received her Ph.D. in computational biology from Tel Aviv University.

Zuben E. Sauna, PhD

Dr. Sauna is a Principal Investigator and a Reviewer at the US Food and Drug Administration. His research interests lie in understanding the pharmacogenetic basis of the immune response to proteins used in therapeutic interventions as these affect efficacy and safety. His laboratory exploits a combination of computational, *in vitro* and *ex vivo* approaches to understand why some individuals and/or sub-populations develop immune responses while others do not. His work has been published in high impact journals such as Nature Biotechnology, Nature Medicine, Science, Science Translational Medicine and Nature Reviews Genetics. He received his Ph.D. from Poona University, India with subsequent training at the National Cancer Institute, Bethesda, USA.

Vahan Simonyan, PhD

Dr. Simonyan has a solid scientific background in varied academic disciplines: MS in Physical Organic Chemistry, Ph.D. in Quantum Physics and Mathematics, post-doctoral training in Nanotechnology and Quantum Statistical Thermodynamics. After 2001, he switched his expertise to biotechnology and biomedical informatics and currently serves at the FDA as a lead scientist of HIVE, R&D Director of Bioinformatics. Vahan is a prolific author of scientific publications in physics, chemistry, quantum chemistry, nanotechnology, biotechnology, population dynamics, and bioinformatics. Additionally, Dr. Simonyan is an adjunct professor at the George Washington University, where he teaches and develops curriculums for biomedical big data informatics and biostatistics research and development courses. His accomplishments in academic and R&D technology carriers have been complemented with the success of technology leadership roles at NCBI and FDA where he established large-scale and complex, science-heavy R&D infrastructures capable of serving worldwide communities for research and regulatory purposes. In 2013, High-performance Integrated Virtual Environment (HIVE) codebase was donated by Dr. Simonyan to the US government in order to build a platform ready to accept NGS data at the US FDA for regulatory review. Today HIVE has supported regulatory review and research leading to peer-reviewed publications in genetics, genomics, proteomics, data modeling, and bioinformatics.

Kyung Sung, PhD

Dr. Sung is a biomedical engineer with expertise in developing functional and practical microscale in vitro tools for medical and biological applications. Dr. Sung's main research interests lie in studying cell-materials interactions and exploring cell behavior in various tissue microenvironmental conditions. Dr. Sung received her Ph.D. in Chemical Engineering in 2007 at the University of Michigan, and worked as a post-doctoral researcher in the Department of Biomedical Engineering at the University of Wisconsin-Madison, where she also worked as a Principal Investigator before she joined the FDA in 2015. She also worked as a patent examiner in Biotechnology at the US Patent and Trademark Office. During her previous research, she used principles from tissue and microsystems engineering to develop tissue-like structures such as blood vessels and mammary ducts in microfluidic channels to develop new practical tools to conduct cancer research in vitro. The microscale in vitro systems provide unique capabilities when studying complex interactions occurring in tissue microenvironment, by providing more precise controls of biochemical and biomechanical factors than traditional platforms. In addition, the small scale of the microfluidic screening platforms allow high-throughput screening experiments using a small number of cells from patient samples and expensive reagents. She has been able to create innovative opportunities and strategies for researchers to explore biology in different ways – particularly in understanding the role of the tissue microenvironment in regulating cellular functions.

John Tsang, PhD

Dr. Tsang leads a laboratory focusing on systems and quantitative immunology at the National Institute of Allergy and Infectious Diseases (NIAID), National Institutes of Health (NIH). He also co-directs the Trans-NIH Center for Human Immunology (CHI) and leads its research program in systems human immunology. Dr. Tsang trained in computer engineering and computer science at the University of Waterloo and received his Ph.D. in biophysics from Harvard University. Before joining the NIH, He worked as a software engineer in Silicon Valley, pursued genomics and proteomics in Rosetta Inpharmatics and Caprion Proteomics, and conducted systems biology and bioinformatics research on microRNAs and integrative genomics at the Massachusetts Institute of Technology (MIT) and Merck Research Laboratories. Dr. Tsang has won several awards for his research, including NIAID Merit Awards for the development of a data reuse and crowdsourcing platform OMiCC and for leading a system biology study of human immune variability and influenza vaccination, which was selected as one of the top 20 NIAID Research Advances of 2014. He has served as a scientific advisor on systems immunology and bioinformatics for a number of programs and organizations including ImmPort (the clinical and molecular data repository for NIAID), the Committee on Precision Medicine for the World Allergy Organization, the NIAID Modeling Immunity for Biodefense Program, the Allen Institute, and the Immuno-Epidemiology Program at the National Cancer Institute.

Carol Weiss, MD, PhD

Dr. Weiss is Chief of the Lab of Immunoregulation in the Division of Viral Products in the Office of Vaccine Research and Review in CBER. Since joining CBER in 1994, her research has focused on mechanisms of virus entry and neutralization, especially for HIV and influenza. Regulatory work includes review of vaccines for HIV and seasonal and pandemic influenza. Her commitment to vaccine research grew out of her experience caring for AIDS patients in the early days of the epidemic during her medical residency in New York City. She received her MD from the University of Chicago and PhD from the University of California, San Francisco.

Hong Yang, PhD

Dr. Yang is a Senior Risk Analyst in CBER's Office of Biostatistics and Epidemiology. She has led many quantitative benefit-risk assessment projects for CBER, and is recognized both within and beyond FDA for her work on risk modeling for transfusion-transmission of infectious diseases such as variant Creutzfeldt-Jakob disease, Zika, and Malaria. She is currently a member of the HHS TTIMS (Transfusion-Transmuted Infections Monitoring System) working group leading development of the analysis plan to monitor HIV incidence among blood donors. Dr. Yang provided advice to WHO and contributed to the WHO guidance Estimation of Residual Risk of Virus Infections in Recovered Plasma/ Blood Components. In 2017, Dr. Yang was invited to present two FDA computational tools for benefit-risk assessment at the WHO global technical consultation meeting "Estimating the Impact of Emerging Infections to the Blood Supply: Requirements for Risk Estimation and Decision Making Support".

Vincent Young, MD, PhD

Dr. Young is the William Henry Fitzbutler Collegiate Professor in the Department of Internal Medicine/Infectious Diseases Division at the University of Michigan Medical School. He received his undergraduate degree from the Massachusetts Institute of Technology and received his M.D. and PhD from Stanford University. He completed his clinical training in Internal Medicine and Infectious Diseases at the Massachusetts General Hospital. He was previously on the faculty at Michigan State University prior to joining the University of Michigan in 2007. Dr. Young has a long-standing interest in understanding the pathogenesis of bacterial infections of the gastrointestinal tract and the role of the normal microbiota in human health and disease. Dr. Young led a Human Microbiome Project on the role of the microbiome in inflammatory bowel disease. He also is involved in projects that look at microbial communities in the lungs of patients with HIV infection and cystic fibrosis. Current research in the Young Lab includes a "team science" effort to understand the pathogenesis *Clostridium difficile* infection by an integrated approach that combines clinical research, bacterial genomics, microbial ecology and immunology/host response projects. He is also leading a group of investigators that is developing the use of stem cell-derived intestinal organoids as a novel alternative model system for the study of enteric disease agents.

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