

# **Biographies**

## **Session I: Introduction**



## Francis S. Collins, M.D., Ph.D.

Dr. Collins is Director of the National Institutes of Health. In that role, he oversees the work of the largest supporter of biomedical research in the world, spanning the spectrum from basic to clinical research.

Dr. Collins is a physician-geneticist noted for his landmark discoveries of disease genes and his leadership of the international Human Genome Project, which culminated in April 2003 with the completion of a finished sequence of the human DNA instruction book. He served as director of the National Human Genome Research Institute at NIH from 1993 to 2008.

Before coming to NIH, Dr. Collins was a Howard Hughes Medical Institute Investigator at the University of Michigan. He is an elected member of the Institute of Medicine and the National Academy of Sciences, was awarded the Presidential Medal of Freedom in November 2007, and received the National Medal of Science in 2009.



## Gary H. Gibbons, M.D.

Dr. Gibbons is Director of the National Heart, Lung, and Blood Institute (NHLBI) at the National Institutes of Health (NIH), where he oversees the third largest institute at NIH, with an annual budget of approximately \$3 billion and a staff of nearly 1,000 federal employees. NHLBI provides global leadership for research, training, and education programs to promote the prevention and treatment of heart, lung, and blood diseases and enhance the health of all individuals so that they can live longer and more fulfilling lives.

Since being named Director of NHLBI, Dr. Gibbons has enhanced the NHLBI investment in fundamental discovery science, steadily increasing the payline and number of awards for established and early-stage investigators. His commitment to nurturing the next generation of scientists is manifest in expanded funding for career development and loan repayment awards

as well as initiatives to facilitate the transition to independent research awards.

Dr. Gibbons provides leadership to advance several NIH initiatives and has made many scientific contributions in the fields of vascular biology, genomic medicine, and the pathogenesis of vascular diseases. His research focuses on investigating the relationships between clinical phenotypes, behavior, molecular interactions, and social determinants on gene expression and their contribution to cardiovascular disease. Dr. Gibbons has received several patents for innovations derived from his research in the fields of vascular biology and the pathogenesis of vascular diseases.

Dr. Gibbons earned his undergraduate degree from Princeton University and graduated magna cum laude from Harvard Medical School. He completed his residency and cardiology fellowship at the Harvard-affiliated Brigham and Women's Hospital in Boston. Dr. Gibbons was a member of the faculty at Stanford University from 1990 to 1996, and at Harvard Medical School from 1996 to 1999.

He joined the Morehouse School of Medicine in 1999, where he served as the founding director of the Cardiovascular Research Institute, chairperson of the Department of Physiology, and professor of physiology and medicine at the Morehouse School of Medicine. While at Morehouse School of Medicine, Dr. Gibbons served as a member of the National Heart, Lung, and Blood Advisory Council from 2009 to 2012.

Throughout his career, Dr. Gibbons has received numerous honors, including election to the Institute of Medicine of the National Academy of Sciences, selection as a Robert Wood Johnson Foundation Minority Faculty Development Awardee, selection as a Pew Foundation Biomedical Scholar, and recognition as an Established Investigator of the American Heart Association (AHA).

#### Scott Gottlieb, M.D.

Dr. Gottlieb was sworn in as the 23rd Commissioner of Food and Drugs on May 10, 2017. Dr. Gottlieb is a physician, medical policy expert, and public health advocate who previously served as the FDA's Deputy Commissioner for Medical and Scientific Affairs and, before that, as a Senior Advisor to the FDA Commissioner. He also worked on implementation of the Medicare drug benefit as a Senior Advisor to the Administrator of the Centers for Medicare & Medicaid Services, where he supported policy work on quality improvement and the agency's coverage process, particularly as it related to new medical technologies.

In 2013, Dr. Gottlieb was appointed by the Senate to serve on the Federal Health Information

Technology Policy Committee, which advises the U.S. Department of Health and Human Services on health care information technology.

Dr. Gottlieb was previously a Resident Fellow at the American Enterprise Institute and a clinical assistant professor at the New York University School of Medicine in Manhattan, where he also practiced medicine as a hospitalist physician. He completed a residency in internal medicine at the Mount Sinai Medical Center and is a graduate of the Mount Sinai School of Medicine and of Wesleyan University, where he studied economics.



#### Peter Marks, M.D., Ph.D.

Dr. Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed an internal medicine residency and hematology/medical oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings, teaching and caring for patients, and in industry on drug development. He joined FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.



## Sally Temple, Ph.D

Dr. Temple is the co-founder and scientific director of the Neural Stem Cell Institute located in Rensselaer, New York. Dr. Temple's group is focused on studies of neural stem cells, using this knowledge to develop therapies for central nervous system disorders.

Dr. Temple trained at Cambridge University and University College London with Dr. Martin Raff, FRS. Her research is focused on the biology of stem cells that give rise to the brain and retina, identifying cell-intrinsic and extracellular niche factors that participate in their self-renewal and differentiation into diverse cell types, and how their characteristics change with aging. Using

patient-derived induced pluripotent stem cells, her research group is building models to study disease mechanisms of agerelated neurodegenerative diseases, with the aim of identifying new targets to slow or stop the disease process. Dr. Temple has received the Royal Society Stothert Research Fellowship, the Javits NIH Merit Award, the MacArthur Award, and the Ellison Investigator Award. Dr. Temple serves on the board and is the immediate past president of the International Society for Stem Cell Research.

## Session II: Musculoskeletal Tissues and Integument



#### B. Lynn Allen-Hoffmann, Ph.D.

Dr. Allen-Hoffmann is Senior Vice President, Regenerative Medicine, Stratatech Corporation, a subsidiary of Mallinckrodt Pharmaceuticals; and tenured full professor, Department of Pathology and Laboratory Medicine and Department of Surgery, University of Wisconsin (UW) School of Medicine and Public Health. Dr. Allen-Hoffmann received her bachelor's degree from the University of Illinois and her Ph.D. from Cornell University. Dr. Allen-Hoffmann was a Damon Runyon-Walter Winchell Postdoctoral Fellow at the Dana-Farber Cancer Institute at Harvard Medical School, where she studied human keratinocyte growth and differentiation.

Dr. Allen-Hoffman is internationally recognized for her innovative research and clinical development of human skin replacements, including the world's first genetically enhanced human skin replacements. The NIKS cells, discovered in her UW laboratory, are a consistent source of genetically uniform, nontumorigenic, pathogen-free human keratinocytes amenable to genetic manipulation. As a result of the discovery in her laboratory, Dr. Allen-Hoffmann founded Stratatech to deliver cell-based therapies to patients with complex skin loss. She has received numerous state and national awards and is the first woman at UW to start a biotechnology company.

Dr. Allen-Hoffmann has contributed to numerous peer-reviewed publications and patents. Dr. Allen-Hoffmann's basic and clinical research has received continuous research support, including grants from NIH, the National Institute of Standards and Technology, the Armed Forces Institute of Regenerative Medicine, the Biomedical Advanced Research and Development Authority (BARDA), and the U.S. Department of Defense.

Recently, Stratatech has been one of the top 10 U.S. companies to receive SBIR and STTR (Small Business Innovation Research and Small Business Technology Transfer) funding. In 2015, BARDA released a solicitation through Project BioShield for the late-stage clinical development and use of cell-based therapies for skin replacement for thermal burns in the event of a mass casualty event resulting from the detonation of an improvised nuclear device. Stratatech was the only academic or private-sector organization in the United States to successfully compete for a BioShield award. The \$247 million Project BioShield award will enable the NIKS technology to be used prior to FDA approval, through the FDA's Pre-Emergency Use Authorization mechanism. Stratatech received a 2016 Tibbitts Award, which Dr. Allen-Hoffmann accepted at a White House ceremony.

Dr. Allen-Hoffmann recently received the Hector F. DeLuca Scientific Achievement Award. Stratatech was acquired by Mallinckrodt Specialty Pharmaceuticals on August 31, 2016.



## Steven R. Bauer, Ph.D.

Dr. Bauer is Chief of the FDA's Cellular and Tissue Therapy Branch (CTTB), Division of Cellular and Gene Therapies (DCGT), Office of Tissues and Advance Therapies (OTAT), Center for Biologics Evaluation and Research (CBER).. 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 Switzerland. He joined CBER's Division of Cellular and Gene Therapies in 1991.



#### Constance Chu, M.D.

Dr. Chu is a professor and the Vice Chair of Research in the Department of Orthopaedic Surgery at Stanford University. She is also the Director of the Joint Preservation Center (JPC) and Chief of Sports Medicine at the VA Palo Alto Health Care System. Previously, she was the Albert Ferguson Professor of Orthopaedic Surgery at the University of Pittsburgh. She is a clinician-scientist who is Principal Investigator of several projects funded by NIH. Dr. Chu has also been recognized as a Castle Connolly/U.S. News and World Report "Top Doctor" in Orthopaedic Surgery as well as on Becker's Spine Review's list of 125 Top Knee Surgeons in the United States. Her clinical practice focuses on the knee: primarily restoration and reconstruction of the ACL, menisci, and cartilage. Dr. Chu graduated from the United States Military Academy at West Point and earned her medical degree from Harvard Medical School.

As Director of the multidisciplinary Joint Preservation Center structured to seamlessly integrate basic, translational, and clinical research with clinical practice, Dr. Chu developed the JPC to advance the concept of early diagnosis and treatment of cartilage injury and degeneration as a strategy to delay or prevent the onset of disabling osteoarthritis. Toward that end, she is leading innovative translational research from bench to bedside in three main areas: quantitative imaging and biomarker development for early diagnosis and staging of joint and cartilage injury and degeneration; cartilage tissue engineering and stem cell-based cartilage repair; and molecular and biological therapies for joint restoration and joint rejuvenation. Her research efforts have led to more than 30 professional awards and honors and include a Kappa Delta Award, considered the highest research honor in orthopaedic surgery.

Dr. Chu also regularly holds leadership and committee positions in major professional organizations such as the American Association of Orthopaedic Surgeons (AAOS) and the American Orthopaedic Association (AOA). In her subspecialty of orthopaedic sports medicine, she is a past President of the Forum Sports Focus Group, a member of the Herodicus Society of leaders in sports medicine, and past Chair of the American Orthopaedic Society for Sports Medicine (AOSSM) Research Council. She is an alumna of the highly regarded AOA American-British-Canadian (ABC) Traveling Fellowship and the AOSSM Traveling Fellowship, opportunities enacted to recognize and promote the careers of emerging leaders in orthopaedic surgery and orthopaedic sports medicine, respectively.



## Joshua Hare, M.D., FACC, FAHA

Dr. Hare is Chief Sciences Officer, Senior Associate Dean for Experimental and Cellular Therapeutics, Director of the Interdisciplinary Stem Cell Institute (ISCI), and Louis Lemberg Professor of Medicine at the University of Miami Miller School of Medicine. Dr. Hare is an expert in cardiovascular medicine and specializes in heart failure, myocardial infarction, inflammatory diseases of the heart, and heart transplantation. He is an internationally acknowledged pioneer in the field of stem cell therapeutics for human heart disease, currently seeing and evaluating patients from all over the world for this new experimental therapy. Dr. Hare has published multiple clinical trials testing the use of mesenchymal stem cells in patients with heart or age-related disorders and is the principal investigator of two major NHLBI programs that advance cell-based therapy.



## Stephen I. Katz, M.D., Ph.D.

Dr. Katz is Director of the National Institute of Arthritis and Musculoskeletal and Skin Diseases, a position he has held since 1995. He was also a Senior Investigator and Chief of the Dermatology Branch of the National Cancer Institute. Dr. Katz has focused his studies on immunology and the skin. He trained many outstanding immunodermatologists in the U.S., Japan, Korea, and Europe. He has served many professional societies in leadership positions, including as Secretary-General of the 18th World Congress of Dermatology in New York in 1992 and as President of both the International League of Dermatological Societies and the International Committee of Dermatology and the Society for Investigative Dermatology. He has received many honors and awards, including the prestigious U.S. Distinguished Executive Presidential Rank Award.



#### Janice S. Lee, D.D.S., M.D.

Dr. Lee has been the clinical director of NIDCR's Division of Intramural Research since 2015. Dr. Lee, a board-certified oral and maxillofacial surgeon, joined NIDCR as the deputy clinical director in August 2013. As chief of the Craniofacial Anomalies and Regenerative Section, Dr. Lee's clinical and translational research program is exploring the natural history and genetic etiology of craniofacial anomalies and growth abnormalities. Her research interests include bone regeneration and stem cell biology, craniofacial congenital anomalies, fibrous dysplasia, and McCune-Albright syndrome. In 2014, Dr. Lee initiated the Craniofacial Anomalies Team and

began to conduct regular case reviews of patients who have craniofacial disorders, as well as provide consultations and treatments to patients at the NIH Clinical Center.

Before joining NIDCR, Dr. Lee was professor of clinical oral and maxillofacial surgery and departmental vice chair in the Department of Oral & Maxillofacial Surgery at the University of California, San Francisco. Dr. Lee's clinical practice focused on craniofacial skeletal reconstruction for people born with cleft lip and palate and other craniofacial anomalies, especially hemifacial microsomia.

Dr. Lee earned a D.D.S. and an M.S. from the University of California, Los Angeles School of Dentistry, and an M.D. from Harvard Medical School. She completed a residency in oral and maxillofacial surgery at Massachusetts General Hospital/Harvard University and a two-year research fellowship in NIDCR's Craniofacial and Skeletal Diseases Branch. Dr. Lee has published nearly 50 peer-reviewed journal articles, dozens of abstracts and poster presentations, and several book chapters.



## Anthony Oro, M.D., Ph.D.

Dr. Oro is the Eugene and Gloria Bauer Professor of Dermatology at Stanford University's Program in Epithelial Biology and a member of the Institute for Stem Cell Biology and Regenerative Medicine, the Stanford Cancer Institute, and the Cancer Biology and Stem Cell graduate student programs. He trained in the medical scientist program at the Salk Institute under Ronald Evans, Ph.D., working on functions of novel orphan nuclear receptors during embryonic development. During his dermatology residency/fellowship in Matthew Scott's lab at Stanford, Dr. Oro helped solidify the first link between the hedgehog pathway, hair follicle development, and human cancer.

In his own lab at Stanford, Dr. Oro uses the skin to address mechanistic question in regenerative medicine, cancer, and autoimmunity. He has a longstanding interest in the mechanisms of hedgehog signaling in the pathogenesis of the most common human tumor, basal cell carcinoma of the skin, recently focusing on tumor evolution and novel resistance-associated signaling pathways. He has identified several novel cancer pathways and focuses on developing inhibitors for treating resistant cancer. Dr. Oro's interest in the mechanisms of human hair and skin development and early ectodermal differentiation has led to understanding hair follicle regeneration and its interaction with the immune system in alopecia areata, as well as the development of therapeutic reprogramming, the use of in vitro human skin differentiation protocols and genome editing tools to produce clinical grade, corrected, autologous human skin from patient-specific induced pluripotent cells. He is focusing his efforts on cell therapies to treat the blistering disease epidermolysis bullosa.

Dr. Oro is a member of the American Society for Clinical Investigation and the American Skin Association. He has received the Marion B. Sulzberger Memorial Award and Lectureship and the William Montagna Lectureship Award.



## Anthony Ratcliffe, Ph.D.

Dr. Ratcliffe is President and CEO of Synthasome, Inc., a biotechnology company in San Diego specializing in orthopedic devices and orthobiologics. Synthasome has an active R&D program with a pipeline of products feeding the company's sales and marketing portfolio, presently focused on tendon and soft tissue repair. Dr. Ratcliffe obtained his B.Sc. in biochemistry in 1977 and his Ph.D. in immunology in 1980 from the University of Birmingham. He then joined the Kennedy Institute for Rheumatology in London as a research scientist; in 1987, he moved to Columbia University in New York as an associate professor of orthopedic biochemistry. In 1996, he joined

Advanced Tissue Sciences (then a leading tissue engineering company), where he served as Vice President for Research until 2002, when he founded Synthasome.

Dr. Ratcliffe has focused on musculoskeletal research, tissue engineering, regenerative medicine, and the translation of these technologies to products. He has served as a member of the Board of Directors of the Orthopaedic Research Society, a member of study sections for NIH, Co-Chairman of the Grant Review Committee for the Orthopaedic Research and Education Foundation, and Co-Chairman of the Tissue Engineered Medical Products Committee for ASTM International. He is a Fellow of the American Institute of Medical and Biological Engineering, has published more than 100 papers, and holds multiple patents.



#### Pamela Gehron Robey, Ph.D.

Dr. Robey is Chief of the Skeletal Biology Section of the National Institute of Dental and Craniofacial Research. She is also the Acting Scientific Director of the NIH Stem Cell Unit.

Dr. Robey has worked in the area of bone for more than 20 years, including work on basic, translational, and clinical studies. She has published about 230 peer-reviewed articles to date. Internationally, she is considered an expert on bone and skeletal stem cell biology.

Dr. Robey has served on numerous editorial boards in the past and is currently the senior editor of *Stem Cell Research* and on the editorial boards of the *Journal of Bone and Mineral Research*, *Stem Cells*, and *Stem Cell Reports*, in addition to being a regular reviewer for many other journals.

She has mentored about 40 research and clinical fellows at various levels of training, the vast majority of whom have progressed in their careers in the field. She is an active member of NIH and the extramural community and focuses in particular on activities to foster career development of junior investigators in the field.



## Dennis Roop, Ph.D.

Dr. Dennis R. Roop is Professor of Dermatology, holds the Charles C. Gates Chair in Regenerative Medicine and Stem Cell Biology, and is director of the Regenerative Medicine and Stem Cell Biology Program at the University of Colorado's Anschutz Medical Campus in Aurora. Prior to joining CU in 2006, Dr. Roop was a professor of molecular and cellular biology at the Baylor College of Medicine in Houston. In 2001, he received the Michael E. DeBakey Award for Excellence in Research, the medical school's highest award. Originally from Jonesville, VA, Dr. Roop graduated from Berea College in 1969 with a degree in biology. He received his MS and PhD degrees from the University of Tennessee, Knoxville, and completed post-doctoral work at

Baylor College of Medicine.



#### Martha J. Somerman, D.D.S., Ph.D.

Dr. Somerman is the Director of the National Institute of Dental and Craniofacial Research (NIDCR), National Institutes of Health. She is also Chief of the Laboratory of Oral Connective Tissue Biology at the National Institute of Arthritis and Musculoskeletal and Skin Diseases. The NIDCR mission is to improve dental, oral, and craniofacial health through research, research training, and dissemination of health information. It is our vision that these activities will be recognized as a catalyst of change in transforming how oral health care is delivered.

Prior to becoming NIDCR's director, Dr. Somerman was dean of the University of Washington School of Dentistry, Seattle, a position she held from 2002 to 2011. Before joining the University of Washington, she was on the faculty of the University of Michigan School of Dentistry, Ann Arbor, from 1991 to 2002, and the University of Maryland from 1984 to 1990. An internationally known researcher and educator, Dr. Somerman's research has focused on defining the key regulators controlling development, maintenance, and regeneration of dental-oral-craniofacial

tissues. She has been a recipient of numerous honors and awards throughout her academic career, including most recently the 2016 American Academy of Periodontology Distinguished Scientist Award.

Dr. Somerman holds a D.D.S. from New York University, a certificate in periodontology from Eastman Dental Center, Rochester, New York, and a Ph.D. in pharmacology from the University of Rochester, School of Medicine and Dentistry.

## Session III: Endocrinology



#### Nancy D. Bridges, M.D.

Dr. Bridges is the Chief of the Transplantation Branch in the Division of Allergy, Immunology, and Transplantation at the National Institute for Allergy and Infectious Disease, NIH. She oversees a portfolio of basic, translational and clinical research in organ and cellular transplantation, with a focus on the immunology of allotransplantation.

Dr. Bridges is a pediatric interventional cardiologist and thoracic organ transplant physician. Before coming to NIH, she held faculty positions at the Children's Hospital, Boston, St. Louis Children's Hospital, the Children's Hospital of Philadelphia, and the Mount Sinai Medical Center in New York City.



#### Bernhard Hering, M.D.

Dr. Hering is internationally renowned for his expertise in islet cell transplantation. His research focuses on finding innovative cell-based therapies to restore blood glucose control and insulin independence for people with type 1 diabetes. Additionally, he is committed to exploring new sources of islet cells through xenotransplantation.

He is the medical director of the Collaborative Islet Transplant Registry (CITR), and has also served as president of the Cell Transplant Society and as councilor of the International Pancreas and Islet Transplant Association.

Dr. Hering also serves on steering committees of major clinical research programs in transplantation and diabetes such as the National Institutes of Health (NIH) Immune Tolerance Network, the Type 1 Diabetes TrialNet, and the Clinical Islet Transplant Consortium, which is the largest ever funded research activity in islet transplantation



#### Mo Heidaran, Ph.D.

Dr. Heidaran is currently a Chemistry, Manufacturing, and Control Master Reviewer at the FDA's Center for Biologics Evaluation and Research (CBER) in the Office of Tissues and Advanced Therapies (OTAT). His in-depth experience in evaluating Investigational New Drugs (INDs) and Biological License Applications (BLAs) comes from both product and facility Current Good Manufacturing Practices (CGMPs) compliance review. He has extensive regulatory knowledge of CGMPs and pre- and post-license inspection of manufacturing facilities for biologics.

Dr. Heidaran has a multidisciplinary academic and industrial background in basic and applied cell biology, innovative cell therapy, and tissue engineering product development. He also has hands-on industrial experience in manufacturing of cell therapy and tissue engineering products. His extensive product development work, manufacturing experience, and comprehensive experience with manufacturing devices allow him to provide a unique insight and perspective in regulatory review and policy making activities. Dr. Heidaran's regulatory work and outreach activities emphasize the importance of defining the critical quality attributes (CQA) and critical process parameters (CPP) for large-scale manufacturing of cellular products. His long-lasting scientific interest is understanding the molecular control mechanisms that regulate growth and differentiation of stem cells in the three-dimensional microenvironments. He is also the founder and first Chair of the prestigious Gordon Research Conference on Signal Transduction by Engineered ECMs.

Dr. Heidaran holds a Ph.D. in biochemistry from the University of South Carolina and received his formal training at the National Cancer Institute. Prior to joining the FDA, he served as R&D Director at both Celgene and Becton, Dickinson and Company. He has been an ad hoc reviewer and member of editorial boards of several peer-reviewed publications. He also holds 25 issued patents and 50 pending patents, and his work has appeared in more than 50 scientific publications.

## José Oberholzer, M.D., M.H.C.M., FACS



Dr. Oberholzer is a professor of surgery and bioengineering, Chief of Transplant Surgery, and Director of the Charles O. Strickler Transplant Center at the University of Virginia. From 2003 to 2017, he has served as Director of the human islet transplantation program, conducting clinical trials of human islet transplantation at the University of Illinois at Chicago. The program has performed more than 600 human islet isolations for both transplantation and research. It is one of six federally funded islet cell resource centers in the United States, providing islet preparations for clinical islet transplant outcomes, expand the number of available human islets, and reduce long-term immunosuppression through comprehensive collaboration with many scientists/physicians in the field of diabetes/islet research and bioengineering. The program has successfully completed a

Phase I/II clinical trial and currently holds three Phase III clinical trials within the Collaborative Islet Transplant Consortium. Currently, Dr. Oberholzer serves as Principal Investigator of several NIH and JDRF International research grants.



## Jon S. Odorico, M.D., FACS

Dr. Odorico is Director of the Pancreas and Islet Cell Transplantation Programs and a tenured professor in the Department of Surgery, Division of Organ Transplantation, at the University of Wisconsin–Madison School of Medicine and Public Health. In addition, he is a research associate at the WiCell Research Institute in Wisconsin. Dr. Odorico received his B.S. in chemistry from Duke University and his M.D. from New York University, and he completed his residency in general surgery as well as a postdoctoral research fellowship, studying islet transplantation and thymic tolerance, at the University of Pennsylvania in Philadelphia. Dr. Odorico is certified by the American Board of Surgery and specializes in pancreatic, islet cell, and multi-organ transplants.

Dr. Odorico has an active research laboratory that focuses on beta cell differentiation from stem cells

for studying pancreas development and for developing novel stem cell-based strategies for treating diabetes. He is the scientific co-founder and Chair of the Scientific Advisory Board of Regenerative Medical Solutions, Inc. (RMS), a biotechnology company based in Madison, WI, developing drug discovery tools and cell therapies for treating diabetes.

Dr. Odorico's research has been supported by NIH, JDRF, the American Diabetes Association, the American Society of Transplant Surgeons (ASTS), and WiCell. He serves as Immediate Past President of the International Pancreas and Islet Transplant Association as well as the Chair of the United Network of Organ Sharing's Pancreas Committee.



## Felicia Pagliuca, Ph.D.

Dr. Pagliuca is a co-founder of Semma Therapeutics and currently serves as Vice President of Cell Biology R&D. Semma is a leading regenerative medicine company, focused on developing a functional cure for diabetes through cell therapy. At Semma, Dr. Pagliuca leads R&D efforts focused on Semma's stem cell technologies, including discovery, process development, and manufacturing. She plays a key role in Semma's preclinical and regulatory strategies for its cell therapy products. Dr. Pagliuca also works closely with Semma's CEO on corporate development activities, including key collaborations, partnerships, and overall strategy.

Previously, Dr. Pagliuca worked with Doug Melton at the Harvard Stem Cell Institute, where she was part of the team that discovered how to generate stem cell-derived beta cells — a discovery that was

named one of the top 10 scientific breakthroughs in 2014. She is an expert in stem cell biology and diabetes and one of the inventors of Semma Therapeutics' key technologies. Dr. Pagliuca received her B.S. from Duke University and her Ph.D. from Cambridge University, where she was a Marshall Scholar. She was previously Director of Technology and Corporate Development at Semma and is currently on leave from Harvard Business School, where she was a Robert S. Kaplan Life Sciences Fellow.



## Klearos Papas, Ph.D.

Dr. Papas has devoted his research career to the application of engineering principles and the development of enabling technologies in the fields of cell therapy and tissue engineering, with a focus on the treatment of diabetes. He has studied and utilized the properties of insulin-secreting tissue (especially as they relate to oxygen demand and supply) and their relationship to viability and function (potency) in the context cell therapies for diabetes, with the objective of improving the cost-effectiveness, availability, practicality, and clinical outcomes of this approach.

Prior to joining the University of Arizona in 2011, Dr. Papas served on the faculty at the University of Minnesota (2003–2011), where he held leadership positions as Associate Director of the Islet Transplant Program, Director of Islet Processing Research and Development, and Director of the Islet Quality Assurance Core in the Schulze Diabetes Institute. Prior to 2011, he held joint research positions at the Massachusetts Institute of Technology in the Department of Chemical

Engineering, the JDRF Center for Islet Transplantation at Harvard Medical School, and the Howard Hughes Medical Institute at Yale University.

Dr. Papas holds a B.Ch.E., an M.S., and a Ph.D. in chemical engineering from the Georgia Institute of Technology, with a focus on tissue engineering, and completed his postdoctoral training at Novartis Pharmaceuticals.

Dr. Papas serves on the council of the Cell Transplant and Regenerative Medicine Society (formerly the Cell Transplantation Society). He also serves on the editorial boards of the journals *Cell Transplantation*, *Cell Medicine*, *Xenotransplantation*, and *CellR4*.



## Daniel Pipeleers, M.D., Ph.D.

Dr. Pipeleers is a member of the Belgian Royal Academy of Medicine. Between 1971 and 1980, he was an investigator for the Research Foundation-Flanders and a Harkness Fellow of the Commonwealth Foundation at the Université libre de Bruxelles (ULB), Washington University in St. Louis, and the Queen Elisabeth Medical Foundation in Brussels.

Since 1980, Dr. Pipeleers has been a professor at the Free University of Brussels (VUB) and University Hospital Brussels. Since 1990, he has been Director of the VUB Diabetes Research Center, where his expertise in beta cell biology, pathology, and therapy guides strategies and studies toward early diagnosis, prevention, and treatment of type 1 diabetes. Dr. Pipeleers is the founder and director of two networks for clinical translation: the Belgian Diabetes Registry, with

the associated Diabetes Biobank Brussels; and the Consortium for Beta Cell Therapy in Diabetes, with support from the European Union and JDRF. The current program on beta cell replacement in type 1 diabetes undertakes (pre-)clinical studies for establishing metabolically adequate beta cell implants using large-scale cell sources, a collaboration with ViaCyte, Inc., in San Diego; the Nestlé Institute of Health Sciences in Lausanne; the San Raffaele Hospital in Milan; Leiden University; and Beta-Cell NV.



## Griffin P. Rodgers, M.D., M.B.A., MACP

Dr. Rodgers was named Director of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) on April 1, 2007. He had served as NIDDK's Acting Director since March 2006 and had been the Institute's Deputy Director since January 2001. As the Director of NIDDK, Dr. Rodgers provides scientific leadership and manages a staff of more than 600 employees and a budget of \$2.0 billion.

Dr. Rodgers received his undergraduate, graduate, and medical degrees from Brown University. He performed his residency and chief residency in internal medicine at Barnes Hospital and the Washington University School of Medicine in St. Louis. His fellowship training in hematology was in a joint program of NIH, the George Washington University, and the Washington DC VA Medical Center. In addition to completing his medical and research training, he earned an M.B.A., with a focus on the business of medicine/science, from Johns Hopkins University in 2005.

As a research investigator, Dr. Rodgers is widely recognized for his contributions to the development of the first effective — and now FDA-approved — therapy for sickle cell anemia. He was a Principal Investigator in clinical trials to develop therapy

for patients with sickle cell disease and also performed basic research that focused on understanding the molecular basis of how certain drugs induce gamma globin gene expression. Recently, Dr. Rodgers and his collaborators have reported on a modified blood stem cell transplant regimen that is highly effective in reversing sickle cell disease in adults and is associated with relatively low toxicity. He has been honored for his research with numerous awards, including the 1998 Richard and Hinda Rosenthal Memorial Award, the 2000 Arthur S. Flemming Award, the 2002 Legacy of Leadership Award, and a 2005 Mastership from the American College of Physicians.

Dr. Rodgers has been an invited professor at medical schools and hospitals, both nationally and internationally. He has been honored with many named lectureships at American medical centers; has published more than 200 original research articles, reviews, and book chapters; has edited four books and monographs; and holds three patents.

Dr. Rodgers is a member of the American Society of Hematology, the American Society for Clinical Investigation, the Association of American Physicians, the American Academy of Arts and Sciences, and the National Academy of Medicine, among others. He served as Governor of the American College of Physicians and as Chair of the Hematology Subspecialty Board and a member of the Board of Directors for the American Board of Internal Medicine.

#### Session IV: Ophthalmology



#### Kapil Bharti, Ph.D.

Dr. Bharti holds a bachelor's degree in biophysics from Panjab University in Chandigarh; a master's degree in biotechnology from the Maharaja Sayajirao University of Baroda; and a diploma in molecular cell biology from Goethe University, where he also obtained his Ph.D., graduating summa cum laude. His Ph.D. work involved research in the areas of heat stress, chaperones, and epigenetics. He did his postdoctoral training at NIH, where he published numerous papers in the areas of transcription regulation, pigment cell biology,

and developmental biology of the eye.

Dr. Bharti has won several awards, including being selected as a Stadtman Tenure-Track Investigator at NIH. His lab was recently awarded two prestigious grants: the only Intramural Common Fund grant to develop a Phase I Investigational New Drug (IND) for autologous, induced, pluripotent, stem cell-derived retinal pigment epithelium tissue, and a U.S. Department of Defense grant to develop a three-dimensional retina tissue to model retinal diseases in vitro. Dr. Bharti's current work as the head of the Unit on Ocular and Stem Cell Translational Research involves understanding mechanism of retinal degenerative diseases by using induced pluripotent stem cell technology and developing cell-based and drug-based therapies for such diseases.



## Dennis Clegg, Ph.D.

Dr. Clegg earned his B.S. in biochemistry at the University of California (UC) Davis and his Ph.D. in biochemistry at UC Berkeley, where he used emerging methods in recombinant DNA to study the sensory transduction systems of bacteria. As a Jane Coffin Childs Memorial Fund Postdoctoral Scholar at UC San Francisco, he studied neural development and regeneration. Dr. Clegg has continued this avenue of research since joining the UC Santa Barbara (UCSB) faculty, with studies of extracellular matrix and integrin function in the developing eye. His current emphasis is on stem cell research, with a focus on developing therapies for ocular disease.

Dr. Clegg is the recipient of the UCSB Distinguished Teaching Award in the Physical Sciences, the Pacific Coast Business Times Champions in Health Care Award, and the National Eye Institute Audacious Goals award. He served as Chair of the Department of

Molecular, Cellular, and Developmental Biology from 2004 to 2009. He has been a Frontiers of Vision Research Lecturer at the National Eye Institute, a keynote lecturer at the Stem Cells World Congress, and a TEDx speaker. Dr. Clegg is the founder and Co-Director of the UCSB Center for Stem Cell Biology and Engineering and has served on advisory boards for the California Institute for Regenerative Medicine and the NIH Center for Regenerative Medicine. He is a Co-Principal Investigator of the California Project to Cure Blindness, a multidisciplinary effort to develop a stem cell therapy for age-related macular degeneration.

# Sophie X. Deng, M.D., Ph.D.



Dr. Deng is a professor at the Stein Eye Institute at the University of California, Los Angeles. She received her M.D. and Ph.D. at the University of Rochester School of Medicine and Dentistry. She completed her residency in ophthalmology at the University of Illinois at Chicago, followed by a fellowship in cornea, external ocular disease, and refractive surgery at the Stein Eye Institute. Dr. Deng specializes in lamellar corneal transplantation, limbal epithelial stem cell deficiency, ocular surface reconstruction, and artificial cornea.

Dr. Deng's group has developed new in vivo imaging techniques and molecular tests to diagnose and stage limbal stem cell deficiency. Dr. Deng's laboratory focuses on developing stem cell– based therapies to treat corneal blindness by regenerating normal corneal tissues from adult stem cells. Current projects include the investigation of Wnt signaling in the regulation of human limbal

epithelial stem cell self-renewal and differentiation, expansion of human corneal endothelial cells, and mechanism of corneal stromal scarring.



#### Donald W. Fink, Jr., Ph.D.

Dr. Fink is in the FDA's Cell Therapy Branch, Division of Cellular and Gene Therapies, Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER). He has nearly 25 years of regulatory review experience.

Presently, Dr. Fink is engaged primarily in regulatory activities pertaining to investigational products composed of or derived from stem cells. He oversees an extensive portfolio of applications that includes hematopoietic, mesenchymal, cord blood, placenta-derived, and pluripotent stem cell (PSC)–derived cellular products. His current appointment is as Expert Regulatory Biologist with specific expertise in the area of human PSCs.

Dr. Fink has organized an FDA advisory committee meeting on the topic of cellular replacement therapies for neurological disorders focused on stem cell–based treatments and has served on the planning committee for an NIH/FDA workshop regarding PSC-based products in clinical translation. He acts as the coordinator for an intra-OTAT working group that monitors development of cellular products derived from PSCs and has served as FDA liaison to both the NIH Stem Task Force and the International Society for Stem Cell Research Task Force on Clinical Translation of Stem Cells.

Dr. Fink is a co-founder and co-chair of an NIH/FDA interagency working group that includes extramural program officers from the National Institute of Neurological Disorders and Stroke established in 2002 to promote cross-agency dialogue to facilitate clinical translation of cellular and gene transfer-based treatments. He has given numerous presentations and authored or co-authored several book chapters describing FDA's approach for evaluation of stem cell–based therapies.



#### David Gamm, M.D., Ph.D.

Dr. Gamm's laboratory studies inherited and acquired eye diseases that culminate in the degeneration of photoreceptors and retinal pigment epithelium (RPE), a significant cause of visual morbidity.

The expansion and targeted differentiation of human stem and progenitor cells in vitro provide an essential source of biological material for modeling retinal development and potential cell-based treatments for these debilitating diseases. The aims of the Gamm laboratory are to investigate cellular and molecular events that occur during retinogenesis and to provide cells for use in rescue or replacement therapies for retinal degenerative diseases.

To meet these goals, Dr. Gamm utilizes a variety of cell types. Human embryonic stem cells (hESCs) are used to delineate the genetic "checkpoints" necessary to produce a particular retinal cell type and serve as a model system for studying human retinal development. Lastly, the Gamm laboratory has directed induced pluripotent stem cells (iPS) towards a retinal lineage in a manner similar to hESCs, allowing for the creation of cell-based models for human retinal degenerative diseases. By understanding the behavior of these cell types in vitro and in vivo, the Gamm laboratory hopes to optimize

strategies to delay or reverse the effects of inherited and acquired eye diseases such as retinitis pigmentosa and macular degeneration.



#### Nitin Gogtay, M.D.

Dr. Gogtay joined the Office of the NIMH Director in June 2013 as Associate Director for Clinical Research, and subsequently assumed the responsibility of Director of the Office of Clinical Research (OCR) for NIMH. He received his M.D. and neuropathology training in India and completed fellowships at the Karolinska Institute, Sydney University, and the National Institute of Neurological Disorders and Stroke (NINDS), later leading a section in the surgical neurology branch. He changed course to pursue research in psychiatry, starting with a second residency at Cornell University. Then he joined the NIMH intramural program where, for more than 15 years, he led the Childhood Onset Schizophrenia Study and the effort to map normal and abnormal brain development from an early age to adulthood. He also serves

as the vice chair of the Intramural Institutional Review Board and serves on FDA's review panels. A critical part of the OCR's responsibilities is to oversee all NIMH-funded human subject research and clinical trials. Dr. Gogtay also serves as a chief advisor to the NIMH director and other senior NIMH leadership about clinical trials design and priorities, and plays an important role in fostering research partnerships between NIMH and other agencies.



#### Derek Hei, Ph.D.

Derek Hei has over twenty years of experience developing breakthrough cell and gene therapies from research through clinical trials. He has expertise in current Good Manufacturing Practices (cGMP) compliance, manufacturing, quality control, quality assurance, and regulatory compliance. Over the course of his career he has overseen the manufacture of cells, DNA, viral vectors, vaccines, and antibodies for clinical use, and for release in numerous global jurisdictions. Dr. Hei joined BlueRock Therapeutics in September 2017 as the Senior Vice President of Manufacturing, Quality and Regulatory. At BlueRock, he is responsible for the strategic planning and management of the company's manufacturing, quality, and regulatory

functions, including its induced pluripotent stem cell platform, cellular therapy production, state-of-the-art cGMP cleanroom facilities, and regulatory filings.

Prior to joining BlueRock, Dr. Hei served as VP of Clinical Manufacture, Quality and Regulatory at Cellular Dynamics International, where he oversaw the development and of its cell therapy products. Dr. Hei also serves as an adjunct professor, Master in Biotechnology program at UW-Madison where he instructs on aspects of biotherapeutic development including process development, scale-up, analytical methods development and key elements of Chemistry, Manufacturing and Control. Previously, Dr. Hei held various roles at Waisman Biomanufacturing, Cerus Corp and Genentech. Dr. Hei earned his Ph.D. in biochemical engineering from the University of California, Berkeley and his bachelor of science in chemical engineering from the University of Wisconsin-Madison.



#### Leonard A. Levin, M.D., Ph.D.

Dr. Levin is Chair of the Department of Ophthalmology at McGill University. He did his undergraduate, graduate, and medical training at Harvard University and then pursued an ophthalmology residency and neuro-ophthalmology fellowship at the Massachusetts Eye and Ear Infirmary. He currently chairs the Executive Scientific Oversight Committee for the Audacious Goals Initiative at the National Eye Institute, which is part of the largest regenerative medicine effort in ophthalmology worldwide. He also chairs the Association of Canadian University Professors of Ophthalmology. He is past Chair of the Diseases and Pathophysiology of the Visual System study section at NIH.

Dr. Levin has published more than 172 peer-reviewed papers, reviews, and book chapters and has four issued patents. He has edited five textbooks in ophthalmology or neuro-ophthalmology, including *Neuro-Ophthalmology: The Practical Guide*, *Ocular Disease: Mechanisms and Management*, and the 11th edition of *Adler's Physiology of the Eye*.

Dr. Levin's basic research is devoted to understanding how diseases of the optic nerve result in loss of connections between the eye and the brain and to finding ways to prevent and reverse that damage. He is also interested in the challenges associated with successfully translating basic science research into clinically effective therapies, particularly for optic nerve disorders, and has been involved in the design and assessment of clinical trials to study neuroprotective therapies in glaucoma and other optic neuropathies.



#### Paul A. Sieving, M.D., Ph.D.

Dr. Sieving directs the National Eye Institute (NEI), part of the National Institutes of Health (NIH). NEI has an annual budget of \$731 million supporting basic, translational, and clinical research projects across the country. In 2013, NEI launched the Audacious Goals Initiative as a 10–15 year strategic effort to develop regenerative therapies for retinal diseases.

Dr. Sieving came to NEI in 2001 from the University of Michigan Medical School, where he was the Paul R. Lichter Professor of Ophthalmic Genetics and the founding director of the Center for Retinal and Macular Degeneration in the Department of Ophthalmology and Visual Sciences. He holds an M.D. and Ph.D. in bioengineering from the University of Illinois. He is an elected member of the National Academy of Medicine and the German National Academy of Science.

Dr. Sieving is known internationally for studies of human retinal neurodegenerative diseases. In 2006, he reported results from the first human clinical trial of ciliary neurotrophic factor for retinitis pigmentosa. In 2015, he initiated a human ocular gene therapy trial for X-linked retinoschisis conducted at the NIH Clinical Center.

#### Session V: Regulatory Considerations for Stem Cell–Based Product Development



## Deborah A. Hursh, Ph.D.

Dr. Hursh is a Senior Investigator and CMC Reviewer in the FDA's Division of Cellular and Gene Therapies, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research (CBER). She received a Ph.D. in molecular, cellular, and developmental biology from Indiana University and did postdoctoral work in the Department of Cellular and Developmental Biology at Harvard University. She was a Senior Staff Fellow in the Laboratory of Biochemistry at the National Cancer Institute and an assistant professor of biology at American University before moving to CBER in 2000. Her expertise is in cell and developmental biology.

At the FDA, Dr. Hursh evaluates a wide range of products relevant to cell and gene therapies and participates in policy development in the areas of stem cells and assisted reproduction. She was Chair of the Humanitarian Device Exemption review committee that approved the Miltenyi Biotec CliniMACS CD34 Reagent System. She chaired the organizing committee for a CBER advisory meeting on oocyte and embryo modification in assisted reproduction for the prevention of transmission of mitochondrial disease. In addition to her regulatory activities, she directs a research lab studying issues relevant to the safety and effectiveness of cell therapy products.



## Martha Lundberg, Ph.D.

Dr. Lundberg earned her Ph.D. in medical sciences from the Texas A&M Health Science Center in the College of Medicine. Her studies were among the first to explore the interplay of extracellular matrix and mechanical forces on vascular cell gene expression and function. In 1995, Dr. Lundberg accepted a National Academy of Sciences Award from the National Research Council, which provided private research support at the National Institute on Aging. Her research focused on the regulation of vascular cell function and the delivery of heart survival signals in cells exposed to oxidative stress. Dr. Lundberg continued this work until joining the National Heart, Lung, and Blood Institute (NHLBI) Division of Cardiovascular Sciences in 2000.

As an NIH Program Director in the Advanced Technologies and Surgery Branch, Dr. Lundberg has proven success in building solid, trusting relationships with key stakeholders to stimulate targeted NHLBI investment in more than a dozen research programs. She brings nearly 18 years of experience and management of cell-based systems for cardiovascular regenerative medicine, including smart polymer systems and biodegradable matrices. She wrote an article entitled "Cardiovascular tissue engineering research support at the National Heart, Lung, and Blood Institute" that was published in *Circulation Research*.

Dr. Lundberg represents the NHLBI and NIH at congressional meetings, national and international scientific conferences, and other trans-governmental activities. She co-chairs the Multi-Agency Tissue Engineering Science Working Group and is

a member of the Bio-Manufacturing USA Federal Stakeholders Council and the HESI global Cell Therapy — TRAcking, Circulation, & Safety Committee, whose mission is to collaborate and share knowledge, experience and resources with an international network of experts in the rapidly evolving field of cell therapy.

Dr. Lundberg has received many distinguished awards as a medical researcher and during her public heath career, including two NIH Director's Awards for her collaboration with the NIH Tissue Chip Project Team and the MATES Federal Strategic Plan, as well as the NHLBI Director's Special Act of Service Awards for work with the National Academy of Sciences Forum on Regenerative Medicine and implementation of the 21st Century Cures Act.



## Michael A. Matthay, M.D.

Dr. Matthay is a professor of medicine and anesthesia at the University of California, San Francisco (UCSF), and a senior associate at the Cardiovascular Research Institute. He is Associate Director of Critical Care Medicine. He received his A.B. from Harvard University and his M.D. from the University of Pennsylvania School of Medicine. Dr. Matthay received clinical training in internal medicine at the University of Colorado and in pulmonary and critical care medicine from UCSF. He also received research training from the Cardiovascular Physiology Laboratory at the University of Colorado and the Cardiovascular Research Institute.

Dr. Matthay's basic research is focused on active ion and water transport mechanisms across the alveolar epithelium that account for the resolution of pulmonary edema, as well as mechanisms

that account for alveolar epithelial repair after acute lung injury and acute respiratory distress syndrome (ARDS). The studies are carried out in both in vivo and in vitro models. Dr. Matthay's clinical research focuses on the mechanisms that account for the pathogenesis and resolution of ARDS and pulmonary edema. In addition, he has studied the biology of bone marrow– derived mesenchymal stem (stromal) cells for the treatment of acute lung injury in both in vitro and in vivo models, as well as testing their potential application in NIH-supported clinical trials of ARDS.



# Steven S. Oh, Ph.D.

As the deputy director of the Division of Cellular and Gene Therapies, Dr. Oh provides leadership in reaching various regulatory decisions on medical products reviewed by the Office of Tissues and Advanced Therapies in the Center for Biologics Evaluation and Research for marketing, clinical investigation, or product classification in the U.S. He is actively involved in the development of regulatory policies for regenerative medicine therapies, including cellular products, gene therapy products, tissue-engineered products, point-of-care devices, and combination products. He interacts closely with the Center for Devices and Radiological Health and the Center for Drug Evaluation and Research in FDA and with other government agencies on cross-cutting scientific and regulatory issues and policies. Dr. Oh participates in international standards activities in the manufacture and testing of cellular products and tissue-engineered medical products. He is also

engaged in various efforts promoting global regulatory convergence in cell and gene therapy products. Before joining FDA in 2007, Dr. Oh served on the faculty of Tufts University School of Medicine in Boston. He was trained as a cell biologist and biochemist at Massachusetts Institute of Technology, Johns Hopkins University School of Medicine, and the University of Michigan.



# Kelley Rogers, Ph.D.

Kelley Rogers is the Technical Program Manager for the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL) at the National Institute of Standards and Technology (NIST) Office of Advanced Manufacturing (OAM). Kelley received a Ph.D. in Molecular Biophysics and Biochemistry from Yale University and a B.A. in Chemistry from Hendrix College.

Kelley is currently on detail from NIST's Material Measurement Laboratory, where she serves as the Technical Program Director for Biosciences and health. In previous positions, Kelley worked as a Principal Investigator identifying novel targets for antimicrobial drugs within the pharmaceutical industry. She was a post-doctoral fellow and staff fellow in the National Institute of Digestive, Diabetes, and Kidney Diseases (NIDDK), at the National Institutes of Health. Kelley's research

background is in bacterial protein synthesis and gene expression.

As the Technical Program Manager, Kelley is responsibility for technical quality and coordination with NIIMBL, a NISTsponsored Manufacturing USA institute whose mission is to accelerate biopharmaceutical manufacturing innovation in the United States.



## Anthony Ting, Ph.D.

Dr. Ting joined Athersys in 2001 as a senior scientist and has been promoted over his 15-year tenure at the company, where he currently serves as Vice President of Regenerative Medicine and Head of Cardiopulmonary Programs. With more than 30 years of experience in cell and stem cell biology, Dr. Ting has developed expertise in translational clinical studies with adult stem cell therapies and has been responsible for all stages of the development of MultiStem<sup>®</sup> from the bench to the bedside. Dr. Ting manages all programs in the cardiovascular and pulmonary areas at the company, as well as the evaluation of potential new uses for the cell therapy product. Dr. Ting serves on several regenerative medicine society committees, including the International Society for Cellular Therapy, the Alliance for Regenerative Medicine, and the American Society for Gene & Cell Therapy.

From 1995 to 2001, Dr. Ting was a Principal Investigator and Head of the Screening for Novel Inhibitors Group at the Institute of Molecular and Cell Biology (IMCB) at the National University of Singapore, where he established a multidisciplinary group that focused on the identification of therapeutic targets and the development and implementation of high-throughput screens. Prior to joining IMCB, he was a Postdoctoral Fellow in the Department of Molecular and Cellular Physiology at Stanford University. Dr. Ting received his Ph.D. in cell biology from Johns Hopkins University and his B.A. in biology from Amherst College.

## Session VI: Neurology



# Scott R. Burger, M.D.

Dr. Burger is the Principal of Advanced Cell and Gene Therapy, a consulting firm specializing in cell and gene therapy product development, manufacturing, and regulatory affairs. Dr. Burger has more than 25 years of experience developing cell and gene therapy products and has consulted for more than 100 industry and academic clients in North America, Europe, Asia, and Australia. He has directed or consulted on process development, manufacturing, and regulatory aspects of a wide range of cell therapy and gene therapy products, including CAR T-cell, NK, and DC immunotherapies; gene-edited cell therapy products; and stem cell– and somatic cell–based regenerative medicine products.

Prior to founding Advanced Cell & Gene Therapy in 2002, Dr. Burger was Vice President for R&D at Merix Bioscience and Director of the University of Minnesota Cell Therapy Clinical Laboratory. His regulatory background includes numerous Investigational New Drug (IND) and Investigational Device Exceptin (IDE) submissions and productive interactions with the FDA Center for Biologics Evaluation and Research's (CBER) Office of Tissues and Advanced Therapies (OTAT).

Dr. Burger is a member of several scientific advisory boards and has served on the U.S. Pharmacopeial Convention (USP) Cell, Gene, and Tissue Therapies Expert Committee, the International Society for Cellular Therapy (ISCT) advisory board, and ISCT committees on gene therapy, regulatory affairs, commercialization, and product/process development. A graduate of the University of Pennsylvania School of Medicine, Dr. Burger completed training in clinical pathology and transfusion medicine at Washington University in St. Louis, is an author on more than 200 scientific publications and presentations, and has received numerous honors and awards.



## Tom Finn, Ph.D.

Dr. Finn is a CMC reviewer in the Division of Cell and Gene Therapies within the Office of Tissues and Advanced Therapies at FDA CBER. He joined FDA in 2006, where he reviews regulatory submissions at all stages of product development, and performs pre-licensure and biannual facility inspections. Dr. Finn reviews a wide range of cellular therapies, including a variety of cancer immunotherapies, and regenerative medicine products such as stem-cell based products for treating cardiovascular or neural conditions. Dr. Finn has regulatory expertise in bioassays, product comparability, product stability, and process validation, and was part of the BLA review team for Provenge and Kymriah. He is involved in numerous CBER internal working

groups, such as CBER's CMC Coordinating Committee, and also serves on the FNIH Biomarker Consortium Neuroscience Steering Committee. He received his Ph.D. in cell biology from Oregon Health & Science University, where he worked on neuronal development; conducted neuroimmunology postdoctoral work at the Portland VA Medical Center, studying multiple sclerosis; and then continued his research interests as a Research Assistant Professor in the Department of Neuroscience at Georgetown University, where he studied stem/progenitor cells in rat models of spinal cord injury.



## Candace Kerr, Ph.D.

Dr. Kerr is the Program Officer of the Stem Cell Program in the Division of Aging Biology at the National Institute on Aging (NIA). NIA's Stem Cell Extramural Program supports major findings and discovery of molecules that facilitate stem cell depletion and cellular senescence, and the relationships with the stem cell niche and aged health. Before joining NIA in 2017, Dr. Kerr was on the faculty of Johns Hopkins University and the University of Maryland School of Medicine, where her laboratory studied human pluripotent stem cells and the translation of those cells to treat spinal cord injury and neurological diseases. Dr. Kerr is the author of many peer-reviewed research articles and is an editor for several journals related to the stem cell biology field.



#### Jane S. Lebkowski, Ph.D.

Dr. Lebkowski has been actively involved in the development of cell and gene therapies since 1986 and is currently President of R&D and Chief Scientific Officer at Asterias Biotherapeutics, Inc., where she is responsible for all R&D of Asterias' products. From 1998 to 2012, Dr. Lebkowski was Senior Vice President of Regenerative Medicine and Chief Scientific Officer at Geron Corporation. Dr. Lebkowski led Geron's human embryonic stem cell program, being responsible for all research, preclinical development, product development, manufacturing, and clinical development activities.

Prior to joining Geron, Dr. Lebkowski was Vice President of Research and Development at Applied Immune Sciences. Following the acquisition of Applied Immune Sciences by Rhone Poulenc Rorer

(RPR, currently Sanofi), Dr. Lebkowski remained at RPR as Vice President of Discovery Research. During her tenure at RPR, she coordinated preclinical investigations of gene therapy approaches for treatment of cancer, cardiovascular disease, and nervous system disorders and directed vector formulations and delivery development. Dr. Lebkowski received her Ph.D. in biochemistry from Princeton University in 1982 and completed a postdoctoral fellowship at the Department of Genetics at Stanford University in 1986.

Dr. Lebkowski has published more than 70 peer reviewed papers and has 13 issued U.S. patents. She has served on the Board of Directors of the American Society of Gene & Cell Therapy and as the Co-Chair of the Industrial Committee of the International Society for Stem Cell Research. Dr. Lebkowski serves on several scientific advisory boards and other professional committees.



#### Robert Mays, Ph.D.

Dr. Mays is the Head of Neurosciences and Vice President of Regenerative Medicine at Athersys. He focuses on the company's novel adult human stem cell product, MultiStem<sup>®</sup>, and its applications in regenerative medicine and drug discovery, with a specific focus on injuries and diseases affecting the central nervous system.

Dr. Mays was the Principal Investigator (PI) of the recently completed MASTERS (MultiStem<sup>®</sup> Administration for Stroke Treatment and Enhanced Recovery Study) clinical trial assessing the safety and efficacy of MultiStem<sup>®</sup> in treatment of ischemic stroke. He will also be the PI of the pivotal Phase III MASTERS-2 study, which has received RMAT (regenerative medicine advanced therapy), fast track, and special protocol assessment designations from the FDA.

Dr. Mays is a member of the National Center for Regenerative Medicine, the Center for Stem Cell and Regenerative Medicine, and the American Heart Association and is an Adjunct Professor at Case Western Reserve University School of Medicine. He has authored or co-authored more than 30 peer-reviewed scientific papers or reviews and holds more than 15 patents relating to the use of stem cells for treating disease. Dr. Mays is on the Commercialization Committee and Patient

Advocacy Committee for the International Society for Cellular Therapy, the Board of Directors for United Cerebral Palsy of Greater Cleveland, and the Scientific Advisory Board for the Children's Neurobiological Solutions Network in Los Angeles.

Dr. Mays graduated from Carnegie Mellon University in 1987 with a B.S. in cell and developmental biology. In 1994, he received his Ph.D. in molecular and cellular physiology at Stanford University. After doing postdoctoral research at the University of Utrecht in the Netherlands; the Weizmann Institute in Rehovot, Israel; and the University of California, San Francisco, Dr. Mays co-founded Athersys, which focuses on developing novel and proprietary best-in-class therapies designed to extend and enhance the quality of human life.



#### David Owens, Ph.D.

Dr. Owens has been the Acting Deputy Director of the Division of Extramural Activities at the National Institute of Neurological Disorders and Stroke (NINDS) since October 2014. Before that, he was a Program Director in the Repair and Plasticity Cluster at NINDS, overseeing a portfolio of grants in stem and progenitor cell biology in the development, function, and repair of the nervous system. He joined the NINDS Division of Extramural Research (DER) as a Program Director in 2004.

Dr. Owens earned a Ph.D. in neurobiology from Columbia University, where he studied cellular signaling during neocortical development, and conducted postdoctoral research in developmental neurobiology at NINDS. Over the last several years, he has been involved in a variety of NINDS and trans-NIH activities

covering a diverse range of topics, including regenerative medicine, cell line authentication, FDA–NIH interactions, piloting of novel funding mechanisms, and promotion of basic neuroscience research.



## Sean I. Savitz, M.D.

Dr. Savitz is a tenured Professor of Neurology; holds the Frank M. Yatsu, M.D. Chair in Neurology; and is the Director of the Institute for Stroke and Cerebrovascular Diseases at McGovern Medical School at the University of Texas Health Science Center at Houston. He graduated from Harvard College, received his M.D. from Albert Einstein College of Medicine, and completed neurology residency training and a cerebrovascular fellowship at the Harvard Medical School Neurology Training Program. He and his team run one of the largest academic stroke programs in the world, testing novel treatments for patients with ischemic stroke and brain hemorrhage.

Dr. Savitz oversees a bidirectional, translational laboratory and clinical research program on cell therapies in stroke and is conducting some of the first clinical trials testing cell therapies in stroke patients. He has been funded by grants from NIH, the Howard Hughes Medical Institute, and the American Heart Association and is an author of more than 100 publications in the biomedical literature.



## Ilyas Singeç, M.D., Ph.D.

Dr. Singeç is the Director of the Stem Cell Translation Laboratory (NIH Regenerative Medicine Program) at the National Center for Advancing Translational Sciences (NCATS). He earned his M.D. and Ph.D. (summa cum laude) from the Universities of Bonn and Freiburg (Germany) and received training in clinical neuropathology. He carried out postdoctoral work at the National Institute of Neurological Disorders and Stroke (NINDS) and the Sanford Burnham Prebys Medical Discovery Institute. He then served as a Laboratory Head and Senior Principal Scientist at Pfizer, where he established a new stem cell laboratory and developed human stem cell–based models for new target

identification and validation for neurological and psychiatric diseases.

Since 2008, Dr. Singeç has generated more than 100 induced pluripotent stem (iPS) cell lines and developed new approaches for controlled cell differentiation and quantitative analysis of pluripotent stem cells. His research interests focus on translating the iPS cell technology into robust and scalable drug discovery and regenerative medicine applications. Dr. Singeç has been the recipient of several scientific awards and fellowships from Pfizer, Merck, the International Bipolar Foundation, the German Research Council (DFG), the California Institute for Regenerative Medicine (CIRM), and NIH.



#### Lorenz P. Studer, M.D.

Dr. Studer is the founding director of the Center for Stem Cell Biology and a member of the Developmental Biology Program at Memorial Sloan Kettering. As a native of Switzerland, he received his medical and doctorate degree from the University of Bern, where he co-developed a fetal cell-based therapy for Parkinson's disease. He subsequently trained as a postdoctoral fellow with Dr. Ron McKay at the National Institutes of Health, pioneering the therapeutic application of neural stem cell-derived neurons in models of neurodegeneration. In his own laboratory in New York, he has established techniques that can turn human pluripotent stem cells into a broad range of cell types of the central and peripheral nervous system, and he

pursued the development of multiple innovative applications in regenerative medicine using human pluripotent-derived cell types. He has also been among the first to realize the potential of patient-specific stem cell in modeling human disease and in drug discovery and developed methods to artificially "age" pluripotent-derived cells for modeling late-onset neurodegenerative disorders. Furthermore, he is currently leading a multidisciplinary consortium to pursue the first in human clinical trials of human stem cell-derived dopamine neurons for the treatment of Parkinson's disease. Dr. Studer's work has been recognized by numerous awards, including the Boyer Young Investigator Award, the Annemarie Opprecht Award, a MacArthur Fellowship, and, most recently, the 2017 Ogawa-Yamanaka prize.



## Su-Chun Zhang, M.D., Ph.D., M.S.

Dr. Zhang is the Steenbock Professor of Behavioral and Neural Sciences in the Department of Neuroscience and Department of Neurology at the University of Wisconsin-Madison. He is also a professor and Director of the Signature Program in Neuroscience and Behavioral Disorders at the Duke-NUS Medical School.

Dr. Zhang received his M.D. and M.S. in China and his Ph.D. in Canada. He has developed a means to guide human stem cells, including induced pluripotent stem cells (iPSCs), to functionally specialized nerve cell types that are lost in many neurological and psychiatric conditions like Parkinson's disease, Huntington's disease, amyotrophic lateral sclerosis (ALS), Alzheimer's disease, and spinal cord injury. He is also conducting cell therapy studies in nonhuman primate models of neurological diseases like Parkinson's disease and spinal cord

injury.

Dr. Zhang has served as a reviewer or consultant for numerous agencies and scientific journals, including NIH, the FDA, and research foundations. He testified to the U.S. Senate on aging and stem cell research. He was a founding member of the WiCell Research Institute.

## Session VII: Hematology



## Andrew P. Byrnes, Ph.D.

Dr. Byrnes is Chief of the Gene Transfer and Immunogenicity Branch in the Division of Cellular and Gene Therapies at FDA's Center for Biologics Evaluation and Research. Dr. Byrnes joined FDA in 2000. He is a principal investigator and reviews manufacturing of gene therapies, cell therapies, and other investigational biologics. Dr. Byrnes has a background in virology and gene therapy, and his laboratory research focuses on adenovirus gene therapy in rodent models. Research interests include vector biodistribution, clearance of vector by the liver, interactions of virions with natural antibodies and complement, and innate immune reactions to viral vectors.



#### Helen Heslop, M.D.

Dr. Heslop is Professor of Medicine and Pediatrics at Baylor College of Medicine and is the Director of the Center for Cell and Gene Therapy at Baylor College of Medicine, Houston Methodist Hospital, and Texas Children's Hospital. She is also Associate Director for Clinical Research at the Dan L. Duncan Cancer Center.

Dr. Heslop is a physician scientist engaged in translational research focusing on adoptive immunotherapy with gene-modified effector cells, to improve hemopoietic stem cell transplantation and cancer therapy. Her initial studies were the first to demonstrate that antigen-

specific cytotoxic T cells could eradicate an established malignancy. Because the cells were genetically marked, she and her collaborators obtained definitive evidence of cell expansion, trafficking to tumor sites, and decade-long persistence. Subsequent protocols have extended this approach to Hodgkin's disease, non-Hodgkin lymphoma (NHL), and nasopharyngeal cancer. She also focuses on reconstituting antiviral immunity post-transplant, and she has led an NHLBI-funded multicenter trial of allogeneic multivirus-specific T cells. She theefore has extensive experience in developing and conducting transplant studies and cell and gene therapy studies and currently holds more than 20 Investigational New Drugs (INDs).

Dr. Heslop was a Doris Duke Charitable Foundation Distinguished Clinical Research Scientist and serves as Principal Investigator on several peer-reviewed research programs, including a National Cancer Institute (NCI)–funded program project grant (Enhancing T-Cell Therapy of Cancer), a Leukemia and Lymphoma Society Specialized Center of Research (SCOR) award (Immunotherapy of Lymphoma), and a Specialized Program of Research Excellence (SPORE) in lymphoma from NCI. She is also the Principal Investigator on an NHLBI-funded training grant in Cell and Gene Therapy. She is the current President of the American Society for Gene and Cell Therapy (ASGCT).



# W. Keith Hoots, M.D.

On January 5, 2009, Dr. Hoots became Director of NHLBI's Division of Blood Diseases and Resources. He had received his A.B. in English and chemistry and his M.D. from the University of North Carolina (UNC) at Chapel Hill. While a senior at UNC, he worked in the hemostasis laboratory of Dr. Kenneth Brinkhous. He then completed his pediatric internship and residency at Children's Medical Center Dallas, Parkland Memorial Hospital. He returned to UNC for his fellowship in pediatric hematology and oncology and worked in the laboratory of Dr. Harold Roberts. Dr. Hoots then joined the faculty at MD Anderson Cancer Center.

Dr. Hoots' major interests involve the management and diagnosis of congenital and acquired bleeding disorders and clotting disorders. His work includes the creation of longitudinal follow-up of hemophilia

cohorts with HIV and hepatitis, gene therapy trials for hemophilia A and B, clinical trials of new clotting concentrates for hemophilias A and B, and the impact of care and clotting factor product on hemophilia patient outcome. He also has a 20-year interest in the diagnosis and treatment of diffuse intravascular coagulation (DIC), particularly in head trauma.

Dr. Hoots has been intimately involved in the development of safe coagulation factor products, having completed his training as the HIV epidemic was evolving in hemophilia patients. By the late 1980s, he was able to return to the hemostasis focus that initially attracted him to the field, and he has continued to be a productive investigator and collaborator. He has a strong interest in global collaborations and in developing public–private partnerships, and he recently completed his sabbatical in Belgium.

Dr. Hoots is a past member of the U.S. Department of Health and Human Services Advisory Committe on Blood and Tissue Safety and Availability, past Chair of the Medical and Scientific Advisory Committee for the National Hemophilia Foundation, and Co-Chair of the DIC Subcommittee of International Society of Thrombosis and Hemostasis, Inc. He has also been an associate editor for *Seminars in Thrombosis and Hemostasis* and served on the editorial boards of *Haemophilia, Haemophilia Forum*, and the *International Monitor on Hemophilia*. Dr. Hoots is a past President of the Hemophilia Research Society of North America.

Dr. Hoots is the NHLBI representative to the Undiagnosed Diseases Network, a Common Fund initiative across NIH. He is a member of the trans-agency research consortium focused on hematologic emergency response for the USA. This is an ongoing program with the U.S. Department of Defense (DoD) and the Biomedical Advanced Research and Development

Authority (BARDA). Dr. Hoots also collaborates with the American Society of Hematology (ASH) to address enhancement of the research and clinical workforce in nonmalignant hematology.

## Linda Kelley, Ph.D.



Dr. Kelley, Cell Therapy Facility Director, is a Senior Member at H. Lee Moffitt Cancer Center & Research Institute and a professor at the University of South Florida. She has provided leadership for cellular therapy facilities for more than 20 years at three institutions: the University of Utah, Dana-Farber Cancer Institute at Harvard University, and Moffitt Cancer Center. She received her graduate and postdoctoral training in immunology and hematology from Vanderbilt University.

Dr. Kelley's scientific career evolved from a fundamental interest in immunological mechanisms of Tlymphocyte function, growth mechanisms of hematopoietic stem and progenitor cells, and molecular changes associated with malignant transformation. Her knowledge of the hematopoietic system led cell biology and therapies

to her interest in stem cell biology and therapies.

As Director of the Cell Therapy Facility at the University of Utah from 1994 to 2011, Dr. Kelley was responsible for developing and expanding the Cell Therapy and Regenerative Medicine Laboratory. During her tenure, she was responsible for preclinical and clinical cell therapy product development to support Investigational New Drug (IND) applications for the production of allogeneic mesenchymal stromal cells (MSCs), autologous bone marrow–derived mononuclear cells, and allogeneic fetal-derived oligodendrocytes. As Director of the Cell Manipulation Core Facility at the Dana-Farber Cancer Institute from 2011 to 2012, Dr. Kelley oversaw management of 20 FDA-approved INDs for the manufacture of genemodified CD34+ cells, tumor cell vaccines, dendritic cells, MSCs, and others. As Director of the Cell Therapy Facility at Moffitt Cancer Center, she oversees 22 active INDs for a variety of cell therapy products, largely to support immunotherapy for adult and pediatric patients. She currently serves as the Principal Investigator for Production Assistance for Cellular Therapies (PACT) — Cell Processing Facilities to perform preclinical cell therapy product development in collaboration with NHLBI and other PACT centers and as Core Laboratory Technical Director for the Moffitt Cancer Center Support Grant. Dr. Kelley excels at bridging the gap between laboratory-based discoveries and new therapies for patients.



## Donald B. Kohn, M.D.

Dr. Kohn is a professor in the Departments of Microbiology, Immunology, & Molecular Genetics (MIMG); Pediatric Hematology/Oncology; and Molecular & Medical Pharmacology at the University of California, Los Angeles (UCLA). He is a board-certified pediatrician with 30 years of experience in clinical bone marrow transplantation.

Dr. Kohn's principal area of research is the development and application of methods for gene therapy of blood cell diseases, such as severe combined immune deficiency (SCID) and sickle cell disease, using autologous hematopoietic stem cells (HSCs). His lab has investigated methods for and expression and more recently for direct gene editing with human HSCs.

optimal gene delivery and expression and, more recently, for direct gene editing with human HSCs.

Dr. Kohn is the sponsor of six investigator-initiated Investigational New Drugs (INDs) for clinical trials of autologous transplant/gene therapy for genetic diseases and pediatric HIV/AIDS. He received a Distinguished Clinical Scientist Award from the Doris Duke Charitable Foundation (2000-2007), is a past President of the American Society of Gene and Cell Therapy (2004) and the Clinical Immunology Society (2014), and was a member and Chair of the NIH Office of Biotechnology Activities (OBA) Recombinant DNA Advisory Committee (RAC) (2010–2015).



#### Harry L. Malech, M.D.

Dr. Malech is Chief of the Genetic Immunotherapy Section, and Deputy Chief of the Laboratory of Clinical Immunology and Microbiology in the National Institute of Allergy and Infectious Diseases (NIAID), National Institutes of Health. Dr. Malech completed his M.D. at Yale University, his residency at the University of Pennsylvania, and his postdoctoral training at the National Cancer Institute and at Yale University. He was an Assistant and Associate Professor of Medicine at Yale University before returning to NIH, where he has been a Senior Investigator since 1986. At NIH, Dr. Malech cares for and studies patients

who have a variety of inherited immune deficiencies, with long-term focus on children and young adults with chronic

granulomatous disease (CGD) or X-linked severe combined immune deficiency (XSCID). His clinical service conducts clinical trials of allogeneic hematopoietic stem cell transplant and autologous stem cell ex vivo lentivector transduction gene therapy for CGD, SCID, and other immune deficiencies. Laboratory research is focused on achieving efficient genetic correction of patient hematopoietic stem cells. Related work includes studies of the generation of induced pluripotent stem

cells from patients with CGD and XSCID, and the use of gene editing methods to genetically correct iPSC or patient hematopoietic stem cells. Dr. Malech is an elected member of the Association of American Physicians and the American Society for Clinical Investigation. He was recent past president for 2014–2015 of the American Society of Gene & Cell Therapy.



#### Kateri Moore, D.V.M.

Dr. Moore is a professor in the Cell, Developmental, and Regenerative Biology Department at the Icahn School of Medicine at Mount Sinai. She is also a member of the Black Family Stem Cell Institute and the Tisch Cancer Institute.

Dr. Moore's work focuses on an understanding of how hematopoietic stem cells (HSCs) self-renew. She led studies that identified and characterized dormant HSCs that sit at the top of the HSC hierarchy, and she is now dissecting the mechanisms that maintain them at the level of DNA and chromatin. Her group was the first to undertake transcription factor–mediated reprogramming of a somatic cell into an HSC without going through pluripotency. These studies have been extended

to human cells and include elucidation of the mechanism by which these factors bind chromatin and initiate a hemogenic program.

Dr. Moore received her doctorate from Kansas State University in 1987 and did postdoctoral studies at Baylor College of Medicine in Houston at what was then the Institute of Molecular Genetics. At Baylor, she did research in the newly developing field of gene therapy, which expanded her interests in HSCs and their microenvironment. In 1992, she went to Princeton University, where she developed a molecular profile of a candidate stem cells niche. She then moved into transgenic model systems to further characterize HSCs and their niche; that came to fruition when she relocated to Mount Sinai in 2007.



#### Robert S. Negrin, M.D.

Dr. Negrin is a professor of medicine and Chief of the Division of Blood and Marrow Transplantation at Stanford University. He received his M.D. from Harvard Medical School and performed his training in internal medicine and hematology at Stanford. He joined the faculty at Stanford in 1990 and was promoted to professor in 2004.

Dr. Negrin's research focuses on developing a more fundamental understanding of graft-versushost and graft-versus-tumor reactions, with a particular interest in immune regulatory mechanisms. He has published more than 240 manuscripts and 42 book chapters. He has served as the President of the International Society of Cellular Therapy and the American Society for Blood and Marrow Transplantation.

Dr. Negrin has won a number of awards, including fellowships from the Damon Runyon-Walter Winchell Cancer Fund and the José Carreras International Leukemia Foundation. He was a recipient of a Distinguished Clinical Science Award from the Doris Duke Charitable Foundation.

Dr. Negrin has served on multiple editorial boards and was an associate editor of *Blood* and the founding editor-in-chief at *Blood Advances*. He is an elected member of the Association of American Physicians.



#### Anne R. Pariser, M.D.

Dr. Pariser is the Deputy Director of the Office of Rare Diseases Research (ORDR) at the National Center for Advancing Translational Sciences (NCATS). ORDR is dedicated to accelerating rare diseases research to benefit patients, through rare diseases programs such as the Rare Diseases Clinical Research Network, the Genetic and Rare Diseases Information Center, and the NCATS Toolkit for Patient-focused Therapy Development.

Dr. Pariser came to NCATS in 2017, after 16 years at the FDA Center for Drug Evaluation and Research (CDER), where she founded the Rare Diseases Program in FDA CDER's Office of New Drugs in 2010. Dr. Pariser earned her M.D. from Georgetown University in Washington, DC, and is board certified in internal medicine. Her research interests include regulatory and translational

science development for rare diseases.



## Robert Sackstein, M.D., Ph.D.

Dr. Sackstein is a professor in the Departments of Dermatology and Medicine at Harvard Medical School and a bone marrow transplant physician at Brigham and Women's Hospital and the Dana-Farber Cancer Institute. Dr. Sackstein also serves as the Director of the Program of Excellence in Glycosciences at Harvard Medical School, as well as the Co-Director of the Harvard University Center for Glycosciences.

Dr. Sackstein received his undergraduate degree in biology, summa cum laude, from Harvard College and his M.D. and Ph.D. from Harvard Medical School, where he also received the James Tolbert Shipley Prize for outstanding research.

Dr. Sackstein's scientific research efforts have helped define the molecular processes that regulate the movement of bloodborne cells into different tissues throughout the body, and clinical applications of his research findings have led to improved outcomes for patients undergoing bone marrow transplantation and for patients with osteoporosis. In particular, Dr. Sackstein is an expert in the structural biology of selectin ligands, and his efforts have led to discovery of a variety of human E-selectin ligands and to development of a glycoengineering technology (known as glycosyltransferase-programmed stereosubstitution or GPS) for steering the trafficking of intravascularly administered cells to marrow, skin, and all sites of inflammation/tissue injury. GPS technology has shown promise in improving the effectiveness of both cell-based regenerative therapeutics and immunotherapeutics.



#### David Scadden, M.D.

Dr. Scadden is the Gerald and Darlene Jordan Professor of Medicine at Harvard University. He is Professor and Chair of the Harvard University Department of Stem Cell and Regenerative Biology. With Douglas Melton, he co-founded and co-directs the Harvard Stem Cell Institute. He is a hematologist/oncologist at Massachusetts General Hospital, where he founded and directs the Center for Regenerative Medicine and previously led the Hematologic Malignancies Program of the MGH Cancer Center. He is a member or fellow of the National Academy of Medicine, the American Academy of Arts and Sciences, the American Association for the Advancement of Science, the American College of Physicians, the Board of External Experts for the National Heart, Lung, and Blood Institute, and a former member of the National Cancer Institute's Board of Scientific Counselors. He is an Affiliate Member of the Broad Institute of Harvard and MIT. He has received

multiple honorary degrees, awards, and memberships in honorary societies. He has published more than 300 articles, has numerous patents, co-founded Fate Therapeutics and Magenta Therapeutics, and is a Director of Agios Pharmaceuticals. His work emphasizes using multidisciplinary approaches to define novel therapies for blood diseases.

#### Session VIII: Cardiology and Vascular Biology



#### James M. Anderson, M.D., Ph.D.

Dr. Anderson is the NIH Deputy Director and Division Director for Program Coordination, Planning, and Strategic Initiatives (DPCPSI) in the Office of the Director. DPCPSI's mission includes identifying emerging scientific opportunities, rising public health challenges, and scientific knowledge gaps that merit further research. The Division plans and coordinates trans-NIH initiatives under 10 overarching themes, as well as overseeing programs supported by the Common Fund, which currently supports the Stem Cell Translation Laboratory within NCATS.

Dr. Anderson received his Ph.D. in biology from Harvard University in 1979, and his M.D. from Harvard Medical School in 1983. Prior to joining NIH, he was Professor and Chair of Physiology at UNC Chapel Hill and Professor of Medicine at Yale. He is an elected member of the American Society for Clinical Investigation, the American Association of Physicians, and a Fellow of the

American Gastroenterological Association. His research in the NHLBI intramural program is focused on understanding epithelial barrier function. He is the recipient of the 2014 Walter B. Cannon Award, the highest research achievement award given by the American Physiological Society.



## Denis Buxton, Ph.D.

Dr. Buxton is the Director of the Basic and Early Translational Research (BETR) Program and Associate Director of the Division of Cardiovascular Sciences at the National Heart, Lung, and Blood Institute (NHLBI). After obtaining his Ph.D. at University College, London, he went to the University of Texas Health Science Center in San Antonio to work on substrate metabolism as a postdoctoral fellow and research instructor. He then joined the faculty at UCLA where, as an associate professor of pharmacology, he studied cardiac ischemia and reperfusion using positron emission tomography. He came to NHLBI via the intramural program, where he worked on signal transduction and nonmuscle myosins. In his current position, he leads the NHLBI Progenitor Cell Translational Consortium and oversees a portfolio of regenerative medicine grants, including Phase I and II

clinical trials in cardiovascular stem cell therapy.



## Karen L. Christman, Ph.D., FAHA

Dr. Christman is a professor in the Department of Bioengineering and the Associate Dean for Students in the Jacobs School of Engineering at the University of California (UC) San Diego. She received her B.S. in biomedical engineering from Northwestern University in 2000 and her Ph.D. from the UC San Francisco and Berkeley Joint Bioengineering Graduate Group, where she examined in situ approaches to myocardial tissue engineering, in 2003. She was also an NIH Postdoctoral Fellow at the University of California, Los Angeles in the fields of polymer chemistry and nanotechnology.

Dr. Christman joined the Department of Bioengineering in 2007 and is a member of the Institute of Engineering in Medicine at UC San Diego. Her lab, which is housed in the Sanford Consortium for Regenerative Medicine, focuses on developing novel biomaterials for tissue engineering and regenerative medicine applications and has a strong translational focus, with the main goal of developing minimally invasive therapies for cardiovascular disease.

Dr. Christman is a Fellow of the American Heart Association and the American Institute for Medical and Biological Engineering and has received several awards, including the NIH Director's New Innovator and Transformative Research Awards, the Wallace H. Coulter Foundation Early Career Translational Research Award, the American Heart Association Western States Affiliate Innovative Sciences Award, and the Tissue Engineering and Regenerative Medicine International Society's Young Investigator Award. Dr. Christman is also co-founder of Ventrix, Inc., which is conducting clinical trials with the cardiac extracellular matrix hydrogel technology developed in her lab at UC San Diego.



## Deborah A. Hursh, Ph.D.

Dr. Hursh is a Senior Investigator and CMC Reviewer in the FDA Division of Cellular and Gene Therapies, Office of Tissues and Advanced Therapies, Center for Biological Evaluation and Research (CBER). She received a Ph.D. in molecular, cellular, and developmental biology from Indiana University and did postdoctoral work in the Department of Cellular and Developmental Biology at Harvard University. She was a Senior Staff Fellow in the Laboratory of Biochemistry at the National Cancer Institute and an assistant professor of biology at American University prior to moving to CBER in 2000. Her expertise is in cell and developmental biology.

At the FDA, Dr. Hursh evaluates a wide range of products relevant to cell and gene therapies and participates in policy development in the areas of stem cells and assisted reproduction. She was chair of the Humanitarian Device Exemption review committee that led to the approval of the Miltenyi Biotec CliniMACS CD34 Reagent System. She was chair of the organizing committee for a CBER advisory meeting on oocyte and embryo modification in assisted reproduction for the prevention of transmission of mitochondrial disease. In addition to her regulatory activities, she directs a research lab studying issues relevant to the safety and effectiveness of cell therapy products.

#### Nicanor I. Moldovan, Ph.D., M.S.

Dr. Moldovan is an associate research professor at Indiana University-Purdue University Indianapolis (IUPUI) and founding Director of the 3D Bioprinting Core at the Indiana University School of Medicine. He received his M.S. in biophysics at the University of Bucharest and his Ph.D. in cell biology from the Institute of Cellular Biology and Pathology "N. Simionescu." Then he pursued postdoctoral training in cardiovascular medicine at Johns Hopkins University.

At the Ohio State University in Columbus Dr. Moldovan performed biomedical (e.g., neovascularization with circulating stem/progenitor cells) and bioengineering (e.g., cellular interaction with polymeric fibrillar scaffolds) research, receiving more than \$4.7 million from NIH (NHLBI and the National Institute on Aging) and the American Heart Association.

Dr. Moldovan's current activity at IUPUI focuses on scaffold-free biofabrication, 3D bioprinting with cell spheroids, and computer modeling of cells and tissues. He equipped the 3D Bioprinting Core Facility, created to support a statewide research program in Indiana, with the Regenova bioprinter by Cyfuse—the first such instrument installed in an academic institution in the United States. The purchase and operation of this scaffold-free bioprinter has been recently funded through a large NIH S10 Shared Instrument Grant, on which he is Principal Investigator. Dr. Moldovan is also an associate editor of the *Journal of Cellular and Molecular Medicine*.



## Lem Moyé, M.D, Ph.D.

Dr. Moyé earned his medical degree at the Indiana University School of Medicine in 1978 and completed a Ph.D. in community health sciences, with a concentration in biostatistics, in 1987. He is a licensed physician in Texas and actively practiced medicine from 1979 to 1992. A diplomate of the National Board of Medical Examiners, he is a teunured professor of biostatistics at the University of Texas School of Public Health in Houston, where he holds a full-time faculty position.

Dr. Moyé has served as a clinical trial consultant with Berlex Laboratories, Procter & Gamble, Marion Merrell Dow, Pfizer, Hoechst Marion Roussel, Aventis Pharma, Key Pharmaceuticals, Coromed, DuPont, Bristol-Myers Squibb, Novartis, Medtronic, AstraZeneca, CryoCor, and Vasogen pharmaceutical companies. He has appeared before the FDA on behalf of several of these sponsors. In addition, Dr. Moyé has served for six years on both the Cardiovascular and Renal

Drugs Advisory Committee to the FDA and the Pharmacy Sciences and Clinical Pharmacology Advisory Committee to the FDA. He has served as an ad hoc member on other FDA advisory committees as well. Dr. Moyé has been an active member of several data and safety monitoring boards that oversee the conduct of clinical trials, and he has taken part in many reviews of grants that have been submitted by fellow scientists for federal funding. He is currently Principal Investigator of the data coordinating center for the NHLBI-funded Cardiovascular Cell Therapy Research Network.

Dr. Moyé has published more than 200 manuscripts in peer-reviewed scientific literature. He has also authored seven books in statistics, including *Statistical Reasoning in Medicine: The Intuitive* P-Value Primer (Springer, 2000 and 2006), *Difference* 

Equations with Public Health Applications (Marcel Dekker, 2000), Multiple Analyses in Clinical Trials: Fundamentals for Investigators (Springer, 2003), Statistical Monitoring of Clinical Trial: Fundamentals for Investigators (2005), Mathematical Statistics with Applications (2005), and Elementary Bayesian Biostatistics (Francis and Taylor, 2007). In addition, he has published a book on professionalism in science (Finding Your Way in Science: How to Combine Character, Compassion, and Productivity in Your Research Career) and a firsthand account of physician responders (Face to Face with Katrina Survivors: A First Responder's Tribute). Dr. Moyé also wrote Saving Grace, a medical thriller.

#### Laura Niklason, M.D., Ph.D.

Dr. Niklason is the Nicholas M. Greene Professor of Anesthesia and Biomedical Engineering at Yale University, where she has been on faculty since 2006. Dr. Niklason's research focuses primarily on regenerative strategies for cardiovascular and lung tissues. Her engineered blood vessels are currently in clinical trials and are the first life-sustaining engineered tissue to be studied in any Phase III trial. Dr. Niklason's lab was also one of the first to describe the engineering of whole lung tissue that could exchange gas in vivo, and this work was cited in 2010 as one of the 50 most important inventions of the year by *Time* magazine. She was inducted into the National Academy of Inventors in 2014 and was elected to the National Academy of Medicine in 2015.

Dr. Niklason received her Ph.D. in biophysics from the University of Chicago and her M.D. from the University of Michigan. She completed her residency training in anesthesia and intensive care

unit medicine at the Massachusetts General Hospital in Boston and completed postdoctoral scientific training at the Massachusetts Institute of Technology.



## Eric Rose, M.D.

Dr. Rose is an academic physician and entrepreneur with interests in drug discovery, biodefense, clinical evaluative research, and health policy. From 2008 to 2013, he chaired the Department of Health Policy at the Mount Sinai School of Medicine. From 1994 through 2007, he served as Surgeon in Chief at New York-Presbyterian Hospital/Columbia and as Chairman of the Department of Surgery at the Columbia University College of Physicians and Surgeons, where he held a distinguished professorship.

An accomplished heart surgeon, researcher, and entrepreneur, Dr. Rose grew one of the nation's premier departments of surgery while managing, investigating, and developing complex medical

technologies ranging from heart transplantation and novel approaches to Alzheimer's disease to bioterrorism. He has authored or co-authored more than 300 scientific publications and has received more than \$25 million in NIH support for his research. Dr. Rose pioneered heart transplantation in children, performing the first successful pediatric heart transplant in 1984, and has investigated many alternatives to heart transplantation, including cross-species transplantation and manmade heart pumps. Siga Technologies has received more than \$100 million in federal research support since he joined the company, developing antiviral drugs for smallpox, dengue, and Lassa fever. He received both his undergraduate and medical degrees from Columbia University.



# Doris A. Taylor, Ph.D., FAHA, FACC

Dr. Taylor is the Director of Regenerative Medicine Research and Director of the Center for Cell and Organ Biotechnology at the Texas Heart Institute (THI) in Houston. She holds faculty appointments at both Texas A&M and Rice University and is a Fellow of the American College of Cardiology, American Heart Association, and the Council on Functional Genomics and Translational Biology, among others.

Dr. Taylor earned her B.S. in biology and physical sciences from Mississippi University for Women in 1977 and earned her Ph.D. in pharmacology from the University of Texas Southwestern Medical Center in 1987. During her postdoctoral studies at Albert Einstein College of Medicine in the Bronx, New York, she first worked with regenerative medicine strategies, growing heart tory and working on gene therapy projects.

muscle cells in the laboratory and working on gene therapy projects.

Before joining THI in 2012, Dr. Taylor directed the Center for Cardiovascular Repair at the University of Minnesota. She also held academic appointments as the Medtronic Bakken Chair of Integrative Biology and Physiology and Professor of Medicine. Dr. Taylor came to the University of Minnesota from Duke University Medical School, where she was on the faculty from 1991 to 2007 and described the first ever functional repair of injured heart with stem cells.

An educator with more than 25 years of teaching experience, she is truly committed to moving innovative therapies from bench to bedside, while preparing students/fellows to compete at an international level in the field of cardiac and vascular repair and regeneration.

Dr. Taylor has published extensively, authoring or co-authoring more than 120 scientific publications. She has been recognized for breakthrough research related to cell therapy, stem cell biology, and tissue engineering-based therapies, as

well as for evaluating differences in male and female stem cells and regenerative medicine strategies. She holds a number of invention disclosures, patent applications, and patents. One of the most notable discoveries laid the groundwork for her research at THI that holds promise for patients with no other option but a heart transplant. In 2008, Dr. Taylor's team published a landmark paper in *Nature Medicine* showing that they could create beating rat hearts using tissue engineering. The lab first stripped the cells away from a rat heart (a process called "decellularization") and then injected rat stem cells into the decellularized rat heart. Dr. Taylor is also conducting research that has uncovered differences in the underlying framework of male and female hearts and other vital organs.

Today, in addition to her research director responsibilities at THI, she co-directs the Cardiovascular Cell Therapy Research Network (CCTRN) Biorepository and leads a cell and cytokine profiling core lab that serves multiple NIH and NHLBI networks, medical centers, and research foundations in the U.S. and Canada.

Dr. Taylor frequently appears as an expert on stem cell therapy and cardiac repair in the media and in the scientific arena. Her work has been recognized and featured since 1998 by 60 Minutes, CNN, *The New York Times, The Wall Street Journal*, BBC Horizon, BBC News Health, ABC News, NBC News, CBS News, Associated Press, National Public Radio, Good Morning America, *Forbes*, the Oprah Winfrey Show, NOVA Science Now, Discovery Channel Through the Worm Hole with Morgan Freeman, Science Channel STEM CELL UNIVERSE with Stephen Hawking, *Huffington Post* (Canada), *The Independent*, and numerous other worldwide media outlets.

From cells to organs, Dr. Taylor is leading international regenerative medicine research efforts, creating cutting-edge therapies for chronic disease, and "building the future treatments of tomorrow — today" at the Texas Heart Institute.

## Session IX: Opportunities in Clinical Trial Design



## llan Irony, M.D.

Dr. Irony is an internist and endocrinologist who joined the FDA Center for Biologics Evaluation and Research (CBER) in September 2000 as a clinical reviewer, after six years in group medical practice in the D.C. area and postdoctoral training at the University of California, San Francisco (UCSF) and NIH. He transferred to the Division of Metabolism and Endocrinology Products in the FDA Center for Drug Evaluation and Research in 2005 and became a Clinical Team Leader in 2009. Since December 2011, he has been at CBER as the Chief of the General Medicine Branch, which is part of the Division of Clinical Evaluation and Pharmacology/Toxicology in the Office of Tissues and Advanced Therapies, and, more recently, as Deputy Division Director.

Dr. Irony wrote or contributed to FDA draft and final guidances, actively participates in scientific and regulatory working groups within and outside the FDA, and interacts with a variety of stakeholders, in addition to regulated industry. He oversees the clinical and the pharmacology/toxicology aspects of regulatory applications for cellular, gene, and plasma-derived products.



## Larissa Lapteva, M.D., M.H.S.

Dr. Lapteva is a clinician with long-standing experience in clinical research investigating novel drugs and biological products. Since joining FDA in 2006, Dr. Lapteva has provided scientific and regulatory advice for clinical development programs with investigational new drugs, generic drugs, and biological products in various therapeutic areas. Dr. Lapteva received her M.D. from Moscow Medical Academy, an M.H.S. from Duke University, and an M.B.A. from the Robert H. Smith School of Business. Dr. Lapteva is serving as the Associate Director in the Division of Clinical Evaluation, Pharmacology, and Toxicology in the Office of Tissues and Advanced Therapies in

the Center for Biologics Evaluation and Research at the FDA.



## Eduardo Marbán, M.D., Ph.D.

Dr. Marbán is an international leader in cardiology and a pioneering heart researcher. His 30plus years of experience in patient care and research have led to key discoveries in gene and stem cell therapies for heart disease. Those discoveries have formed the basis for multiple startup companies.

Dr. Marbán attended public schools through high school and later Wilkes College, where he earned a B.S. in mathematics before completing a combined M.D.-Ph.D. program at Yale

University. Postgraduate training took him to the Osler Medical Residency Training Program at Johns Hopkins University, where he spent 26 productive years. During his tenure, he served in a variety of academic and research leadership positions, including as Chief of Cardiology.

In his research career, Dr. Marbán, a cellular electrophysiologist by training, has pursued questions relevant to heart disease (ischemia, heart failure, and arrhythmias). His laboratory elucidated the fundamental pathogenesis of myocardial stunning, pioneered the concept of gene therapy to alter electrical excitability, and created the first de novo biological pacemaker as an alternative to electronic pacemakers. Dr. Marbán first became interested in stem cells in 2002, building upon his work on biological pacemakers. Since 2004, his lab has been intensively studying cardiac progenitor cells, their origins, and their therapeutic potential. The basic work has come full circle, in that Dr. Marbán's cardiac-derived cell products form the basis for six grant-funded clinical trials: two completed (CADUCEUS and DYNAMIC) and four ongoing (ALLSTAR, HOPE-Duchenne, Regress-HFpEF, and ALPHA).

In 2007, Dr. Marbán became founding Director of the Cedars-Sinai Heart Institute, a multidisciplinary entity that brings together adult and pediatric cardiologists, cardiac surgeons, imaging specialists, and researchers to foster discovery and enhance patient care. The institute is built on a long tradition of excellence and innovation at Cedars-Sinai, including the invention of the Swan-Ganz catheter. The Cedars-Sinai Heart Institute, ranked by *US News and World Report* as the top heart program in the western United States, performs more heart transplants annually than any other institution worldwide.

Among the many honors Dr. Marbán has received are the Basic Research Prize of the American Heart Association (AHA), the Research Achievement Award of the International Society for Heart Research, the Gill Heart Institute Translational Award, and the Distinguished Scientist Awards of the AHA and the American College of Cardiology.



# Anne R. Pariser, M.D.

Dr. Pariser is the Deputy Director of the Office of Rare Diseases Research (ORDR) at the National Center for Advancing Translational Sciences (NCATS). ORDR is dedicated to accelerating rare diseases research to benefit patients, through rare diseases programs such as the Rare Diseases Clinical Research Network, the Genetic and Rare Diseases Information Center, and the NCATS Toolkit for Patient-focused Therapy Development.

Dr. Pariser came to NCATS in 2017, after 16 years at the FDA Center for Drug Evaluation and Research (CDER), where she founded the Rare Diseases Program in FDA CDER's Office of New Drugs in 2010. She earned her M.D. from Georgetown University and is board certified in internal medicine. Her research interests include regulatory and translational science development for rare diseases.



#### Sean I. Savitz, M.D.

Dr. Savitz is a tenured professor of neurology, holds the Frank M. Yatsu, M.D. Chair in Neurology, and is the Director of the Institute for Stroke and Cerebrovascular Diseases at McGovern Medical School at the University of Texas Health Science Center at Houston. He graduated from Harvard College, received his M.D. from Albert Einstein College of Medicine, and completed neurology residency training and a cerebrovascular fellowship at the Harvard Medical School Neurology Training Program. He and his team run one of the largest academic stroke programs in the world, testing novel treatments for patients with ischemic stroke and brain hemorrhage.

Dr. Savitz oversees a bidirectional, translational laboratory and clinical research program on cell therapies in stroke and is conducting some of the first clinical trials testing cell therapies in stroke patients. He has been funded by grants from NIH, the Howard Hughes Medical Institute, and the American Heart Association and is an author on more than 100 publications in the biomedical literature.

## Session X: A Path to Treatments and Cures

#### Rachael Anatol, Ph.D.

Dr. Anatol is Deputy Director of the Office of Tissues and Advanced Therapies (OTAT) within the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration (FDA). Prior to this role, Dr. Anatol served as an Associate Director of Policy in OTAT (formerly the Office of Cellular, Tissue, and Gene Therapies). Dr. Anatol received her Ph.D. in molecular and cell biology from the University of Maryland, College Park, and completed her postdoctoral training at the National Heart, Lung, and Blood Institute.



#### Thomas Bollenbach, Ph.D.

Dr. Bollenbach is the Chief Technology Officer of the Advanced Tissue Biofabrication Manufacturing Innovation Institute (ATB-MII). Prior to joining the ATB-MII, he was Vice President for Research and Development at Harvard Apparatus Regenerative Technology (now Biostage), where he was responsible for the development and execution of Investigational New Drug (IND)–enabling preclinical and product development programs for implantable airway and esophageal constructs containing living cells.

Before working at HART, Dr. Bollenbach was Associate Director of Preclinical Research and Development at Organogenesis Inc., where he implemented and completed IND-enabling efficacy and preclinical pharmacology and toxicology programs for living, bioengineered skin grafts. He earned a Ph.D. in biochemistry at the University of Notre Dame.



#### Ruben G. Carbonell, Ph.D.

Dr. Carbonell is the Frank Hawkins Kenan Distinguished Professor of Chemical and Biomolecular Engineering at North Carolina (NC) State University. He is the Chief Technology Officer of the National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL), a recently launched Manufacturing Innovation Institute in the Manufacturing USA network. Dr. Carbonell is on temporary leave as Director of the Biomanufacturing Training and Education Center (BTEC), a position he has held since 2008. He has also been the Director of the Kenan Institute for Engineering, Technology & Science since 1999. He served as head of the Department of Chemical Engineering at NC State from 1995 to 1999.

Dr. Carbonell was elected to the National Academy of Engineering in 2014 for the impact of his work on molecular recognition applied to biological separations and on transport processes. He is a Fellow of the National Academy of Inventors, the American Institute of Chemical Engineers, and the Industrial and Engineering Chemistry Division of the American Chemical Society. Dr. Carbonell is a Foreign Member of the Slovenian Academy of Sciences and Arts and the Academy of Sciences of the Institute of Bologna. He has won numerous awards, including the Holladay Medal for Excellence at NC State — the highest award given to university faculty.

Dr. Carbonell received his B.S. in chemical engineering from Manhattan College in 1969 and his Ph.D. in the same area from Princeton University in 1973. During his academic career, he has supervised more than 100 M.S., Ph.D., and postdoctoral students, many of whom hold leading positions in industry and academia. Dr. Carbonell has served on numerous academic and industrial boards and has participated in and helped to organize many national and international meetings.



#### Joshua Hare, M.D., FACC, FAHA

Dr. Hare is Chief Sciences Officer, Senior Associate Dean for Experimental and Cellular Therapeutics, Director of the Interdisciplinary Stem Cell Institute (ISCI), and Louis Lemberg Professor of Medicine at the University of Miami Miller School of Medicine. Dr. Hare is an expert in cardiovascular medicine and specializes in heart failure, myocardial infarction, inflammatory diseases of the heart, and heart transplantation. He is an internationally acknowledged pioneer in the field of stem cell therapeutics for human heart disease, currently seeing and evaluating patients from all over the world for this new experimental therapy. Dr. Hare has published multiple clinical trials testing the use of mesenchymal stem cells in patients with heart or age-related disorders and is the principal investigator of two major NHLBI programs that advance cell-based therapy.

#### Janet Lynch Lambert, M.B.A.



Ms. Lambert joined the Alliance for Regenerative Medicine (ARM) in 2017 as the organization's first CEO. With more than 25 years in public- and private-sector management, she is an experienced government relations and business professional with an extensive record of accomplishment. She most recently served as the Acting Head of Engagement for the *All of Us* Research Program at NIH and as head of the Outreach Office in the Office of the NIH Director.

Prior to joining NIH, Ms. Lambert was Vice President of Government Relations and head of the Washington office of Life Technologies, aiding the company in its growth from \$300 million in annual sales to more than \$3 billion. Prior to joining Life Technologies, she held leadership positions in government relations, marketing, and business development at large and small life science organizations, including GE Healthcare Life Sciences and InforMax, Inc. Her experience also includes legislative and staff

organizations, including GE Healthcare Life Sciences and InforMax, Inc. Her experience also includes legislative and staff leadership positions in the U.S. Senate and House of Representatives.

Ms. Lambert received her M.B.A. in international business from Georgetown University and her B.A. in political science from Stanford University.



#### Peter Marks, M.D., Ph.D.

Dr. Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed an internal medicine residency and hematology/medical oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings, teaching and caring for patients, and in industry on drug development. He joined FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.



## José Oberholzer, M.D., M.H.C.M., FACS

Dr. Oberholzer is a professor of surgery and bioengineering, Chief of Transplant Surgery, and Director of the Charles O. Strickler Transplant Center at the University of Virginia. From 2003 to 2017, he has served as Director of the human islet transplantation program, conducting clinical trials of human islet transplantation at the University of Illinois at Chicago. The program has performed more than 600 human islet isolations for both transplantation and research. It is one of six federally funded islet cell resource centers in the United States, providing islet preparations for clinical searchers throughout the world. Dr. Oberholzer's research strives to improve clinical islet transplant outcomes, expand the number of available human islets, and reduce long-term immunosuppression through comprehensive collaboration with many scientists/physicians in the field of diabetes/islet research and bioengineering. The program has successfully completed a

Phase I/II clinical trial and currently holds three Phase III clinical trials within the Collaborative Islet Transplant Consortium. Currently, Dr. Oberholzer serves as Principal Investigator of several NIH and JDRF International research grants.



#### Anthony Oro, M.D., Ph.D.

Dr. Oro is the Eugene and Gloria Bauer Professor of Dermatology at Stanford University's Program in Epithelial Biology and a member of the Institute for Stem Cell Biology and Regenerative Medicine, the Stanford Cancer Institute, and the Cancer Biology and Stem Cell graduate student programs. He trained in the medical scientist program at the Salk Institute under Ronald Evans, working on functions of novel orphan nuclear receptors during embryonic development. During his dermatology residency/fellowship in Matthew Scott's lab at Stanford, Dr. Oro helped solidify the first link between the hedgehog pathway, hair follicle development, and human cancer.

In his own lab at Stanford, Dr. Oro uses the skin to address mechanistic question in regenerative medicine, cancer, and autoimmunity. He has a longstanding interest in the mechanisms of hedgehog signaling in the pathogenesis of the most common human tumor, basal cell carcinoma of the skin, recently focusing on tumor evolution and novel resistance-associated signaling pathways. He has identified several novel cancer pathways and focuses on developing inhibitors for

treating resistant cancer. Dr. Oro's interest in the mechanisms of human hair and skin development and early ectodermal differentiation has led to understanding hair follicle regeneration and its interaction with the immune system in alopecia areata, as well as the development of therapeutic reprogramming, the use of in vitro human skin differentiation protocols and genome editing tools to produce clinical-grade, corrected, autologous human skin from patient-specific induced pluripotent cells. He is focusing his efforts on cell therapies to treat the blistering disease epidermolysis bullosa.

Dr. Oro is a member of the American Society for Clinical Investigation and the American Skin Association and has received the Marion B. Sulzberger Memorial Award and Lectureship and the William Montagna Lectureship Award.



#### Amy P. Patterson, M.D.

Dr. Patterson is the Chief Science Advisor and Director of Scientific Research Programs, Policy, and Strategic Initiatives at the National Heart Lung and Blood Institute of the National Institutes of Health. In this capacity, she provides oversight and coordination of trans-NHLBI projects and manages a broad portfolio of issues germane to the conduct of clinical research, research oversight, programmatic planning, policy development, new scientific initiatives, and relationships with organizations within and external to the Institute. Prior to joining NHLBI, Dr. Patterson served as the NIH Associate Director for Science Policy. Dr. Patterson has also served as Deputy Director of the Division of Cellular and Gene Therapies and Senior Medical Officer in the Division of Clinical Trials Design and Analysis at the Center for Biologics Evaluation

and Research at the U.S. Food and Drug Administration.

Dr. Patterson received a B.A. from Harvard University and an M.D. from the Albert Einstein College of Medicine. She completed residency training in internal medicine at the Memorial Sloan Kettering Cancer Center and Cornell Medical School, New York Hospital, where she also served as Assistant Chief Resident. She completed clinical and basic research fellowships in endocrinology and metabolism at the National Institute of Diabetes and Digestive and Kidney Diseases and in the disorders of lipoprotein metabolism at NHLBI. She has won many distinguished service awards during her public health career, including the NIH Director's Awards of Merit, FDA Commissioner's Special Citations, and the U.S. Secretary of Health's Distinguished Service Award.



## Sally Temple, Ph.D.

Dr. Temple is the co-founder and scientific director of the Neural Stem Cell Institute located in Rensselaer, New York. Dr. Temple's group is focused on studies of neural stem cells, using this knowledge to develop therapies for central nervous system disorders.

Dr. Temple trained at Cambridge University and University College London with Dr. Martin Raff, FRS. Her research is focused on the biology of stem cells that give rise to the brain and retina, identifying cell-intrinsic and extracellular niche factors that participate in their self-renewal and differentiation into diverse cell types, and how their characteristics change with aging. Using

patient-derived induced pluripotent stem cells, her research group is building models to study disease mechanisms of agerelated neurodegenerative diseases, with the aim of identifying new targets to slow or stop the disease process. Dr. Temple has received the Royal Society Stothert Research Fellowship, the Javits NIH Merit Award, the MacArthur Award, and the Ellison Investigator Award. Dr. Temple serves on the board and is the immediate past president of the International Society for Stem Cell Research.