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Committee Listing

Co-Chairs
Francesca Cook, MPH
REGENXBIO
Rockville, MD

Morrie Ruffin
Alliance for Regenerative Medicine
Washington, DC

Members
Anne-Virginie L. Eggimann, MS
bluebird bio
Cambridge, MA

Lynne Fahey McGrath, MPH, PhD
REGENXBIO
Rockville, MD

Adam Roose
Alliance for Regenerative Medicine
Washington, DC

Commercialization Workshop Supporters

The American Society of Gene & Cell Therapy is honored to acknowledge the following organizations for their support of the Commercialization Workshop:
## Program

### 8:30 AM – 9:30 AM

**Keynote Address**
*SPEAKER:* John Furey, Spark Therapeutics

### 9:30 AM – 10:30 AM

**Value Frameworks for Gene and Cell Therapy**  
*Moderator:* Michael Werner, JD, Alliance for Regenerative Medicine

*SPEAKERS:*  
- Sarah Emond, MPP, Institute for Clinical and Economic Review  
- Phil Reilly, MD, JD, Third Rock Ventures  
- Colleen Rye, PhD, FasterCures  
- Marianne Hamilton Lopez, PhD, MPA, Duke-Robert J. Margolis, MD, Center for Health Policy

### 10:30 AM – 11 AM

**Coffee Break**

### 11 AM – 12 PM

**Regulatory Advances**  
*Moderator:* Anne-Virginie Eggimann, bluebird bio

*SPEAKERS:*  
- Peter Marks, MD, PhD, Center for Biologics Evaluation and Research, FDA  
- Snehal Naik, PhD, Janssen Research & Development, LLC  
- Robert Pietrusko, Pharm.D, Voyager Therapeutics Inc.  
- Lynne McGrath, PhD, REGENXBIO

### 12 PM – 1 PM

**Lunch**

### 1 PM – 2 PM

**Patient Voice**  
*Moderator:* Jennifer Bernstein, Horizon Government Affairs

*SPEAKERS:*  
- Stephen M. Rose, PhD, Foundation Fighting Blindness  
- Melissa Hogan, JD, Project Alive  
- Laurice Levine, MA, CCLS, Children's Los Angeles, Thalassemia Support Foundation  
- Jennifer Farmer, MS, CGC, Friedreich's Ataxia Research Alliance

### 2 PM – 3 PM

**Real World Market Access Panel**  
*Moderator:* Kristin Viswanathan Wolff, MPH, bluebird bio

*SPEAKERS:*  
- Sarah Pitluck, MSc, Spark Therapeutics  
- Mark Trusheim, MSc, MIT NEWDIGS, FoCUS Project  
- Rocio Manghani, Kite, a Gilead Company  
- Bill Martin, Accredo Health
3 PM – 3:30 PM

Coffee break

3:30 PM – 4:30 PM

CMC Panel

MODERATOR: Stewart Abbot, PhD, Fate Therapeutics

SPEAKERS: Zhenhong Li, PhD, REGENXBIO
Jerry Keybl, MilliporeSigma
Bitao Liang, PhD, Celgene
Peter Olagunju, bluebird bio
Xin Swanson, PhD, MBA

4:30 PM – 5:30 PM

Industry Perspectives

MODERATOR: Morrie Ruffin, Alliance for Regenerative Medicine

SPEAKERS: Jonathan Garen, uniQure
Barrie J. Carter, PhD, BioMarin Pharmaceutical
Donna Armentano, PhD, Pfizer Inc.
Amy DuRoss, MBA, Vineti
Faculty Bios

Stewart Abbot, PhD  
Fate Therapeutics

Dr. Abbot is Chief Development Officer at Fate Therapeutics and is leading the company’s early product development and translational science initiatives. Previously, Dr. Abbot held senior research roles at Celgene Cellular Therapeutics (CCT). As Senior Director of Research at CCT his group developed novel therapeutic candidates based on hematopoietic stem cells and human placenta-derived cells and initiated clinical trials for placental cells. As Executive Director of Integrative Research at CCT he led Integrative Research activities for CCT that encompassed technology and product scouting, alliance identification and business development activities, including initiation of engineered T cell programs. Prior to CCT, Dr. Abbot led General Electric’s Molecular and Cellular Biology research laboratory at its Global Research Center in Albany, NY, where he established GE’s expertise in human stem cell biology and developed a series of life science products and instruments. Dr. Abbot holds a B.Sc. in Biological Sciences (Edinburgh), M.Sc. in Biomedical Engineering (Glasgow) and Ph.D. in Pathology (London).

Jennifer Bernstein  
Horizon Government Affairs

Jennifer joined Horizon Government Affairs in December 2008 as Vice President, focusing on legislative and regulatory developments within the pharmaceutical, biotechnology and medical device sectors. Prior to joining Horizon, Jennifer was Vice President of Healthcare Research at a mid-sized healthcare advisory and financial services firm. In this capacity, she managed over 100 clients, including hedge funds, mutual funds, investment advisers and healthcare corporations and was responsible for anticipating and translating legislative and regulatory catalysts within all sectors of the healthcare marketplace. Jennifer also spent several years on Capitol Hill as a legislative assistant to former Congressman James Greenwood (R-PA). Prior to that, Jennifer served as professional staff on the Judiciary Committee in the Pennsylvania House of Representatives, where she helped to develop Megan’s Law. She has held the Series 7 (General Securities Representative), Series 65 (Uniform Investment Advisor) and Series 87 (Registered Research Analyst) securities licenses.

Jennifer received her Bachelor of Arts in Political Science and History from Millersville University of Pennsylvania, and Master’s Degrees in both American Government and International Relations from Temple University.

Francesca Cook, MPH  
REGENXBIO

Ms. Cook has over 25 years of experience in healthcare policy and reimbursement. She is currently Senior Director, Pricing and Market Access at REGENXBIO responsible for global pricing and market access strategy and activities across the company’s therapeutic disease areas. Previously, she served as Senior Vice President, Policy & Government Affairs and Program Management at PharmAthene, Inc. Prior to her tenure at PharmAthene, Ms. Cook served in senior management positions overseeing policy and reimbursement at Guilford Pharmaceuticals Inc. and Covance Health Economics and Outcomes Services. Before joining Covance she served as Legislative Assistant in the U.S. Senate and worked at the U.S. Department of Health and Human Services. Ms. Cook holds a Master of Public Health degree from Yale University School of Medicine Department of Epidemiology and Public Health and a Bachelor of Arts degree in Biology from Mount Holyoke College.

Anne-Virginie L. Eggimann, MS  
bluebird bio

Anne-Virginie joined bluebird bio in September 2011 to lead the Regulatory Science function. Prior to joining bluebird bio, Anne-Virginie was an Executive Director at Voisin Consulting, leading projects involving the design and implantation of regulatory strategies for medicinal products, with a particular focus on rare diseases including oncology, and advanced therapies. Her experience spans from early development through commercialization including lead roles on the registration of several orphan drugs and regulatory expertise on both sides of the Atlantic. Anne-Virginie holds a Master of Science in Environmental Health Sciences from the UCLA School of Public Health, as well as a B.S. in Chemical Engineering from the California Institute of Technology.

Sarah K. Emond, MPP  
Institute for Clinical and Economic Review

With nearly 20 years of experience in the business and policy of healthcare, Sarah leads the strategic planning and operations of the Institute for Clinical and Economic Review, a leading non-profit health policy research organization, as Executive Vice President and Chief Operating Officer. In that role, she is responsible for overseeing ICER’s public programs, communications, operations, and finances.

Prior to joining ICER, Sarah spent time as a communications consultant, with six years in the corporate communications and investor relations department at a commercial-stage biopharmaceutical company, and several years with a healthcare communications firm. Sarah began her healthcare career in clinical research at Beth Israel Deaconess Medical Center in Boston. A graduate of the Heller School for Social Policy and Management at Brandeis University, Sarah holds a Master of Public Policy degree with a concentration in health policy. Sarah also received a bachelor’s degree in biological sciences from Smith College.

Jennifer Farmer, MS, CGC  
Friedreich's Ataxia Research Alliance

Jennifer Farmer is the Executive Director of the Friedreich’s Ataxia Research Alliance, having joined the organization in 2006 and been ED since 2008. Jennifer has a Master’s degree in Genetic Counseling and prior to joining FARA she worked at the University of Pennsylvania and Children's Hospital of Philadelphia. As a genetic counselor at the University of Pennsylvania Jennifer helped to establish the Division of Medical Genetics, focusing on adult genetics, and later became the Division Administrator. Jennifer’s
Faculty Bios – continued

developed a special interest in neurogenetic conditions and then went on to establish and coordinate clinical and research programs for individuals and families diagnosed with Friedreich Ataxia (FA) and related neurodegenerative diseases. In her current role at FARAs as Executive Director, she helps to carry out the strategic mission of the organization through administering FARAs research grant, scientific conference, patient registry, and education and awareness programs.

**John Furey**  
**Spark Therapeutics**

John Furey is responsible for global commercial operations, medical affairs, technology development and technical operations at Spark Therapeutics. He has 25 years of experience in developing and implementing operational strategies and leading commercial and technical teams.

Prior to joining Spark Therapeutics, John was senior vice president and head of global operations for Baxalta, where he directed manufacturing, quality, engineering, and process development. He actively managed a $2.5 billion production budget across Baxalta's global network and led a first-in-class supply chain organization for rare diseases. John led the team that coordinated and delivered the successful establishment of Baxalta through a spin out from Baxter and led the Baxter Vaccine inline business to realize significant top line and bottom line growth. He also spent two years in China as general manager of Pfizer's vaccine business unit following a role with responsibility for global pricing and reimbursement at Pfizer Vaccines. In these roles, John gained extensive experience in pipeline development and global product launches. Earlier in his career, he held both commercial and operations positions of increasing scope and responsibility with Pfizer and Wyeth Pharmaceuticals.

John has an executive M.B.A. from St. Joseph's University, Philadelphia, a B.S. from Trinity College, Dublin, and a diploma in Environmental Health from the Dublin Institute of Technology.

**Jonathan Garen**  
**uniQure**

Mr. Jonathan Garen joined uniQure as Chief Business Officer in July 2016. Most recently, Mr. Garen served as Chief Business Officer at Syros Pharmaceuticals, where he was responsible for business transactions including partnering Syros' technology platform and drug assets, and bringing in products to enhance and accelerate its pipeline. Prior to joining Syros, Mr. Garen was the Assistant Vice President of Business Development at Forest Laboratories from 2003 to 2014, and subsequently, Actavis, plc until 2015 following its acquisition of Forest Laboratories. At Forest Laboratories and Actavis, Mr. Garen was responsible for numerous acquisitions and license agreements to build the companies' pipeline in all its focus therapeutic areas, and led a team of business development professionals. Earlier in his career, Mr. Garen was Director of Global Licensing with Pharmacia Corporation and a Founder and Vice President of Technology Exchange, Inc., in New York, NY. Mr. Garen holds a Master of Environmental Science degree from Yale University and a Bachelor of Science degree in Physics from the Massachusetts Institute of Technology.

**Marianne Hamilton Lopez, PhD, MPA**  
**Duke-Robert J. Margolis, MD, Center for Health Policy**

Marianne Hamilton Lopez, PhD, MPA is Research Director of the Value-Based Payment Reform portfolio at Duke-Margolis. In this role, she manages the Center's activities aimed at identifying barriers and facilitating implementation of new value-based payment models for pharmaceuticals, including gene therapies, and medical devices. She oversees the Developing a Path to Value-Based Reimbursement for Medical Products Consortium and partners with Duke University faculty, scholars, and external health experts to advance this work.

Prior to joining Duke-Margolis, Dr. Hamilton Lopez was a senior program officer with the National Academy of Medicine's Leadership Consortium for a Value & Science-Driven Health System and provided strategic direction and oversight of the Consortium's Science and Technology portfolio and Clinical Effectiveness Research Innovation and the Digital Learning Collaboratives. She was a Senior Manager at AcademyHealth; a Public Health Community Advisor for the United States Cochrane Center; and the Federal Women's Program Manager and American Indian/Alaska Native Employment Program Manager for the National Institutes of Health.

**Melissa Hogan, JD**  
**Project Alive**

A former corporate attorney and strategy consultant, Melissa's life changed when her son Case was diagnosed with Hunter Syndrome (MPS II) in 2009. She now serves as the President of Project Alive, a leading Hunter Syndrome research and advocacy foundation and also acts as an FDA Patient Representative, an advisory board member for the Mayo Clinic Social Media Network, and on the Corporate and Foundation Alliances for Global Genes. Melissa is considered a leading expert on clinical trials for neurodegenerative diseases and has consulted on the design of clinical trials for Hunter Syndrome around the world.

In her work with Project Alive, Melissa has spearheaded creative and award-winning campaigns for Hunter Syndrome awareness and fundraising, including viral videos, a documentary series, and even an original song she wrote called “Alive.” Project Alive is currently funding and anticipating 2018 enrollment of a Phase I/II gene therapy clinical trial for Hunter Syndrome. The site for the study, Nationwide Children's Hospital in Columbus, Ohio, received IND approval in late 2017.

Melissa is also currently writing her second book on pediatric medical trauma, as well as a separate book about patient advocacy.

**Jerry Keybl**  
**MilliporeSigma**

Jerry Keybl heads the Cell & Gene Therapy Manufacturing Franchise at MilliporeSigma. Based in Bedford, MA, Jerry responsible for virus & gene therapy manufacturing services as well as cell & gene therapy manufacturing products and process
Faculty Bios – continued

development services. Jerry has held a number of strategy and marketing roles over his career. Jerry holds a PhD in Chemical Engineering from MIT and two bachelor degrees from the University of Pennsylvania in Chemical Engineering and Finance (Wharton).

Rocio Manghani
Kite, a Gilead Company
Rocio Manghani is the Sr. Director of Payer Relations at Kite, A Gilead Company.
Kite is focused on the development and commercialization of novel cancer immunotherapy products designed to harness the power of a patient’s own immune system to eradicate cancer cells.

Mrs. Manghani has extensive experience in health policy and determining the impact to the commercialization of biologics with orphan status. With over 15 years of biopharmaceutical experience, she is a recognized expert on public and private payer policy. Rocio’s responsibilities at Kite includes overseeing the development and execution of Kite’s payer and reimbursement strategies for its CAR-T therapy. Prior to joining Kite, Mrs. Manghani was at Celgene Corporation where she was the Director of Regional Accounts. During her tenure at Celgene, she developed and implemented payer strategies for all Celgene therapeutic areas and was an integral part of the health policy development team. Previously, she held management positions at Roche Pharmaceuticals and Abraxis Bioscience, which helped to fuel her passion of ensuring patients have access to the right medications at the right time. Mrs. Manghani earned her Master’s in Public Health from Emory University and her undergraduate degree from the University of California, San Diego.

Peter Marks, MD, PhD
Center for Biologics Evaluation and Research, FDA
Peter Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed 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 the FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.

Bill Martin
Accredo Health, an Express Scripts Company
Bill Martin is the Chief Commercial Officer for Accredo, an Express Scripts Company. In this role, Mr. Martin is responsible for guiding the development and growth of the organizations overall specialty business with payers, pharmaceutical manufacturers, and distribution partners. He leads a team that is heavily focused on growth through strategic initiatives, innovative clinical programs, and market development efforts.

Mr. Martin previously served as Vice President of Business Development and Strategy for Accredo and has more than 25 years of experience within the healthcare industry. Mr. Martin has held various positions of leadership including general management, marketing, medical education, sales, and product development. His extensive industry experience includes pharmaceuticals, biologics, medical devices, transplants, clinical trials, and distribution network management.

His key areas of interest include working with clients to maximize the value of their pharmacy and medical benefits, as well as helping manufacturers to navigate the very complex and confusing waters of the specialty marketplace. Mr. Martin holds a bachelor’s degree in Communications and Business from The University of Tennessee.

Laurice Levine, MA, CCLS
Children’s Los Angeles, Thalassemia Support Foundation
Laurice Levine has her Bachelors of Science in Human Development and Education from University of California at Davis and her Master’s degree in Child and Family Studies from Syracuse University, New York. After she completed her education she earned her Child Life Certification which licenses her to do specialized clinical and psychosocial work with children in hospitals. Laurice worked professionally as a thalassemia outreach coordinator and advocate for 17 years. Currently, Laurice is an Independent medical consultant, working with bluebird biotech in Boston, Massachusetts, and Children’s Hospital Los Angeles, California. Laurice is also a current board member of the Thalassemia Support Foundation. Finally, Laurice is a 45-year old with thalassemia major. She has been happily married for 13 years and is the mother to their 2-year old son, 10-year-old Corgi and 3 birds. Laurice enjoys traveling, music and movies and is a Disney aficionado.

Zhenhong Li, PhD
REGENXBIO
Dr. Zhenhong Li is a highly accomplished technical and quality executive with over 28 years of intense, successful and hands on experience in Biotechnology and Pharmaceuticals Industry. She has a Ph.D. in Chemistry from Princeton University and then went on got further trainings in cellular signal transductions associated with tyrosine kinases at the National Institutes of Health and Bristol Myers Squibb during her post-doctoral years. Throughout her career, she has worked at Bristol Myers Squibb, Gilead Sciences, Medimmune, Baxter Biosciences, Human Genome Sciences, GSK and REGENXBIO in the fields of small molecules, nucleotides, protein therapeutics, vaccines and gene therapy. Her expertise are at strategically harnessing cross functional talents and technological advancements and applying the effective and objective analytical technologies in product characterization, production support and quality control operations.

Bitao Liang, PhD
Celgene
Bitao Liang, Ph.D. is the Head of Product Science, Cell Therapy Development at Celgene Corp. Prior to current position, he has served as the CMC lead of Celgene/Juno CAR T collaboration since 2016. He had also held a few positions in research and discovery function at Celgene Cellular Therapy Division from 2007 to 2015. He had led a team and advanced multiple research projects to
Faculty Bios – continued

development, including mesenchymal stromal cell and T cell based product pipelines for oncology and immunology applications. He had also led non-clinical development efforts to support IND submissions. Dr. Liang obtained a Ph.D. degree in Immunology from New York Medical College, Valhalla, NY and also a Bachelor of Science degree in biochemistry from East China University of Science and Technology, Shanghai, China.

Lynne Fahey McGrath, MPH, PhD
REGENXBIO
Lynne Fahey McGrath, MPH, Ph.D., is the Vice President of Regulatory Affairs at REGENXBIO, a leading AAV gene therapy focused clinical-stage biotechnology company. In addition, she serves on the Scientific Advisory Committee for BioPontis Alliance for Rare Diseases. Prior to joining REGENXBIO in 2015, Dr. McGrath spent 3 years as an R&D global leader for Novartis Consumer Health and 8 years at Novartis Oncology managing global strategy for multiple development teams, accelerating approval of multiple drugs including several for rare diseases. As a member of the executive team for Novartis Oncology US organization, she participated in multiple business-critical endeavors, e.g., compliance initiatives, pharmacovigilance, due diligence and oversight of promotional material review and approval. Prior to Novartis, Dr. McGrath spent over 15 years leading regulatory teams developing global products for primary care, oncology and rare genetic diseases. Dr. McGrath holds a PhD and an MPH in public health from the University of Medicine and Dentistry of NJ - Robert Wood Johnson Medical School, where her research focused on the use of innovative scientific discoveries to inform regulatory decision making through effective risk analysis. In addition, she received a B.S. in biology from the University of Connecticut.

Snehal Naik, PhD
The Janssen Pharmaceutical Companies of Johnson & Johnson
Snehal brings a confluence of early discovery, innovation and scientific expertise to her current role Associate Director and North America regulatory liaison supporting cell and gene therapies, microbiome and vaccines at Janssen, the pharmaceutical division of Johnson & Johnson. With a keen interest in influencing regulatory policies in fields of regenerative medicine and advanced therapies, she staffs the regulatory committee and gene therapy section of the Alliance for Regenerative Medicine, and is an active member of the American Society of Gene & Cell Therapy and New York Academy of Sciences. With a penchant for exploring the unexplored, she has led biology programs at both Pfizer’s Centers for Therapeutic Innovation and Johnson & Johnson’s Janssen Incubator. Snehal holds an AB-MA in Biology from Bryn Mawr College and a Ph.D. in Molecular Genetics and Genomics from Washington University in St. Louis.

Robert G. Pietrusko, Pharm.D.
Voyager Therapeutics, Inc.
Robert G. Pietrusko, Pharm.D. is Senior Vice President, Regulatory Affairs & QA at Voyager Therapeutics, Inc. He has three decades of experience in regulatory affairs and pharmaceutical drug development across a wide range of therapeutic categories including CNS disorders and products governed by the Center for Biologics Evaluation and Research (CBER) of FDA. Dr. Pietrusko has directed the worldwide approval of more than 30 new products and new indications and is a recognized leader in the pharmaceutical industry having served as a BIO representative for PDUFA III and IV reauthorization negotiations with FDA. He also developed the concept of an expedited review program for gene therapy products and drafted initial legislative language for the 21st Century Cures Act proposed by the Alliance for Regenerative Medicine that eventually led to the Regenerative Medicine Advanced Therapy (RMAT) designation process. Before joining Voyager, Dr. Pietrusko held executive and senior level positions at ViroPharma, Inc., Millennium Pharmaceuticals and SmithKline Beecham (now Glaxo SmithKline).

Dr. Pietrusko holds a Bachelor of Science degree in biology and a Bachelor of Pharmacy degree from Rutgers University and a Doctor of Pharmacy degree from the Philadelphia College of Pharmacy and Science. He completed his residency training in hospital pharmacy at Thomas Jefferson University Hospital. Dr. Pietrusko is the author or co-author of 52 scientific publications.

Sarah Pitluck, MSc
Spark Therapeutics
Ms. Pitluck is the Head of Global Pricing & Reimbursement (P&R) at Spark Therapeutics, Inc., a fully integrated gene therapy company committed to meeting the needs of patients living with genetic diseases. She has been involved with healthcare market access issues including coverage, coding, and global payment systems for 20 years.

In her current role, Ms. Pitluck successfully priced the first US Food and Drug Administration-approved gene therapy for a genetic disease, LUXTURNA™ (voretigene neparvovec-rzyl). She is now working to ensure market access for all US patients in need of LUXTURNA including completing the first Centers for Medicare & Medicaid Services (CMS) demonstration project to provide alternate payment options and value-based arrangements for single-administration therapies like LUXTURNA. She is also responsible for conducting global pricing and health economic and outcomes assessments for additional products in Spark’s pipeline and for chairing Spark’s P&R Committee, which is responsible for all high-level strategic pricing, reimbursement, and market access decisions at the company.

Prior to joining Spark, Ms. Pitluck was the Executive Director for Global P&R at Alexion Pharmaceuticals, where she led all global P&R efforts, including dossier preparation and negotiations for Alexion’s products worldwide. Ms. Pitluck was formerly the Director of the Coverage & Reimbursement Policy group within Genentech’s Government Affairs division where she was responsible for a team focused on all US public payer policy and reimbursement issues for Genentech’s products. Before Genentech, she worked at Avalere Health, a policy consulting firm, where she focused on conducting reimbursement analyses for a variety of pharmaceutical, biotechnology, and medical device products in a wide range of care settings and disease areas. She
Faculty Bios – continued

also worked at Covance Health Economics and Outcomes Services Inc. where she specialized in interviewing physician thought-leaders, coding experts, and medical directors of national and regional health plans to advise clients on market access solutions. Ms. Pitluck began her career at the Institute of Medicine writing reports with national experts for Congress on various health policy issues.

Ms. Pitluck received her Bachelor of Arts degree from Washington University in St. Louis, Missouri and her Masters of Science at the London School of Economics and Political Science.

Philip R. Reilly, MD, JD
Third Rock Ventures
Philip R. Reilly (Cornell AB ’69, Columbia JD ’73, Yale MD ’81) is a Venture Partner at Third Rock Ventures in Boston, Massachusetts. Trained in internal medicine and clinical genetics, he specializes in starting and launching companies to develop breakthrough therapies for heretofore untreatable genetic disorders. Among other roles, he has served on the boards of Edimer Pharmaceuticals and Lotus Tissue Repair, Inc. In 2010 he helped to found bluebird bio Inc, a gene therapy company, and from 2010 to 2012 he served as its Chief Medical Officer. More recently, Reilly has been a founder of Voyager Therapeutics (which specializes in using the adeno-associated virus to treat disorders of the central nervous system) and Fulcrum Therapeutics, a company that will focus on gene regulation. Prior to joining Third Rock Ventures, Dr. Reilly was (2000 - 2006) CEO and Chairman the Board of Interleukin Genetics, Inc. Prior to joining Interleukin, Dr. Reilly was (1990-2000) the Executive Director of the Eunice Kennedy Shriver Center for Mental Retardation, Inc., a not-for-profit research organization dedicated to helping people with developmental disabilities, and an Assistant Professor at Harvard Medical School. Dr. Reilly served twice (2000 and 2003) as President of the American Society of Law, Medicine, and Ethics. From 1994-1997, he served on the Board of Directors of the American Society for Human Genetics. He is a founding fellow of the American College of Medical Genetics. Dr. Reilly was a trustee of Cornell University from 2006-2014. He is currently a member of the Board of Overseers of Weill Cornell Medical College. He also serves on the Advisory Council to the BU School of Public Health. He is the author of seven books about human genetics and more than 100 articles, many about public policy issues in genetics. His latest (seventh) book, Orphan: The Quest to Save Children with Rare Genetic Disorders, was published on November 1, 2015.

Stephen M. Rose, PhD
Foundation Fighting Blindness
Dr. Rose joined the Foundation Fighting Blindness as the Chief Scientific Officer in December 2004. He manages and oversees the day-to-day operations of the Science Department, work closely with the Foundation’s Scientific Advisory Board and Research Oversight Committee, and provides overall leadership to its funding program. In addition, Dr. Rose works to establish a seamless pipeline of science and clinical studies to move developments and treatments into clinical trials while partnering with pharma and biotech to maximize potential commercialization.

Prior to joining the Foundation, Dr. Rose served Director, Division of Clinical Recombinant DNA Research, Office of Biotechnology Activities (OBA), NIH Office of Science Policy. In this position, he served as the Executive Secretary for the Recombinant DNA Advisory Committee and provided oversight and coordination for the recombinant DNA program to address issues regarding recombinant DNA, including human gene transfer clinical protocols.

Before joining OBA, Dr. Rose was Director of the Office of Clinical Applications in the Division of Allergy, Immunology and Transplantation at the National Institute of Allergy and Infectious Diseases. Before accepting that position, Dr. Rose was the Chief of the Transplantation Immunobiology Branch in the same NIAID Division and established the kidney transplant clinical trial program.

Dr. Rose received his B.S. in Biology with Honors from American University and his Ph.D. from the University of Virginia. Upon completion of his doctorate, he was a NIH postdoctoral fellow at Washington University in St. Louis. He also held academic positions at the University of Texas Southwestern Medical School and the University of New Mexico Cancer Research Center. Dr. Rose currently sits on the FDA’s Cellular, Tissue and Gene Therapies Advisory Committee and is a Health Research Alliance Board member.

Colleen Rye, PhD
FasterCures
Colleen Rye, PhD is a Director at FasterCures. Prior to joining FasterCures, Rye was Chief of Army Virtual Health, where she led a global telemedicine organization across 30 countries and territories, 18 time zones, and more than 50 clinical specialties. Rye also led telehealth strategy and collaboration efforts across Air Force, Army, and Navy and with the Department of Veterans Affairs, and she was instrumental in the creation of the military’s first virtual medical center providing care across garrison and combat zones. Prior to Army, she was a health care investment banker with Raymond James and Associates and SunTrust Equitable Securities, and she volunteered with the peace and health teams at The Carter Center. Rye earned a PhD and a MS from The Wharton School, University of Pennsylvania and a BA from the University of the South (Sewanee), where she served on the Board of Trustees and currently serves on the Cabinet for the Campaign for Sewanee. Her awards include two Department of the Army Meritorious Civilian Service Awards, and she is a Distinguished Member of the United States Army Medical Department Regiment.

Morrie Ruffin
Alliance for Regenerative Medicine
Mr. Ruffin has more than 25 years of experience in the Biotech and Healthcare industries. He is a co-founder of the Alliance for Regenerative Medicine (ARM), the global organization representing the interests of the regenerative medicine.
Faculty Bios – continued

community. He continues to support ARM as a senior advisor. Mr. Ruffin also played a leading role in establishing the Standards Coordinating Body for Regenerative Medicine and most recently led the effort to launch the ARM Foundation for Cell and Gene Medicine. He currently serves on the Board of the ARM Foundation and as the organization’s Executive Director. Morrie is also the managing partner of Adjuvant Partners, a boutique regenerative medicine and advanced therapies business development and strategic advisory firm. Prior to joining Adjuvant Partners, he was the chief executive officer of LifeTech Innovations, LLC a business development consulting firm based in Bethesda, MD. Prior to his position at LTI, Mr. Ruffin was executive vice president of Capital Formation and Business Development at the Biotechnology Industry Organization (BIO), the largest trade organization representing the biotech and drug development industries. Joining BIO in 1994 as one of its original employees, Mr. Ruffin was responsible for building the organization’s global business development and investor outreach programs focused on helping companies raise capital and identify strategic partnering and licensing opportunities. This BIO business development franchise is now one of the largest in the world, with operations in the US, Europe, and Japan.

In addition to his business development work at BIO, Mr. Ruffin was responsible for leading the industry’s capital formation advocacy efforts with a focus on economic incentives to promote investment in early stage biotech and med-tech businesses. He was also a founder and board member of the Interoperable Informatics Infrastructure Consortium (IIC), an international standards setting body for the bioinformatics industry. Prior to joining BIO, Mr. Ruffin worked for US Senator Arlen Specter for five years as his senior legislative assistant. Prior to that, he spent approximately five years working in varying capacities, including policy analyst at Systems Planning Corporation International and the Center for Strategic and International Studies. Mr. Ruffin received his M.A. in International Studies & Economics from Johns Hopkins School for Advanced International Studies (SAIS) and his B.A. from the University of Virginia.

Mark Trusheim, MSc
MIT NEWDIGS, FoCUS Project

Mark Trusheim is a Visiting Scientist at the MIT Sloan School of Management and Strategic Director, NEWDIGS at the MIT Center for Biomedical Innovation. He is the Founder and President of CoBio Consulting, LLC and has been a Special Government Employee for the FDA’s Office of the Commissioner.

Mark’s research focuses on the economics of biomedical innovation, especially precision medicine, adaptive pathways, platform trials and their enablement by digital health advances. He also studies biotechnology regional clusters. His work emphasizes using quantitative modeling in multi-stakeholder processes to inform public policy, corporate strategy, and product development and commercialization.

Prior to MIT, his career spanned big data and simulations at Kenan Systems, marketing at Searle Pharmaceuticals, eHealth as Vice President of Monsanto Health Solutions, genomics as President of Monsanto’s Cereon Genomics, molecular diagnostics as CEO of start-up Cantata Laboratories and policy as the President of the Massachusetts Biotechnology Council. Mark was a member of the Monsanto Pipeline Management Team and led the Monsanto Bioinformatics/IT activities.

Mark is a frequent invited speaker on precision medicine, companion diagnostics, adaptive pathways and regional life sciences development at international conferences. He holds degrees in Chemistry from Stanford University and Management (Finance and IT) from MIT.

Kristin Viswanathan Wolff, MPH
bluebird bio

Kristin Viswanathan Wolff joined bluebird bio in 2017 as the Director, Global Government Affairs and Policy. bluebird is a clinical stage biopharmaceutical company headquartered in Cambridge, Massachusetts, focused on developing cell and gene therapies for severe genetic diseases and cancer. In this role, Kristin is responsible for working to shape the regulatory landscape in target markets to enable patient access to bluebird gene therapies once approved and to better ensure that reimbursement systems take into account the value our therapies can provide to patients and healthcare systems over the short and long term. Prior to joining bluebird, Kristin held the role of Director, Reimbursement & Health Policy at the Biotechnology Innovation Organization (BIO), a trade association representing R&D-intensive, innovative biopharmaceutical companies, headquartered in Washington, D.C.

At BIO, her portfolio included traditional coding, coverage, and reimbursement policy under Medicare and Medicaid, as well as issues related to the implementation of the Affordable Care Act (ACA) and the Medicare Access and CHIP Reauthorization Act (MACRA). At BIO, Kristin led workstreams specifically related to value-based payment arrangements and value assessment frameworks in the U.S. Kristin came to BIO in 2012 from the National Academy of Medicine (NAM) (formerly the Institute of Medicine) having supported expert publications authored on behalf of the NAM Forums on Neuroscience and Nervous System Disorders, and Medical and Public Health Preparedness for Catastrophic Events. Kristin holds a B.A. in Neuroscience from the Johns Hopkins University and an MPH from Dartmouth College.

Michael Werner, JD
Alliance for Regenerative Medicine

Michael Werner has almost three decades of healthcare law, lobbying, regulatory, reimbursement and policy development experience in Washington. He is the co-founder and executive director of ARM as well as a Partner at Holland & Knight LLP. In these roles, Michael focuses on issues affecting biotechnology and pharmaceutical companies, medical research and research institutions, physicians and patients. His specific areas of knowledge include legislation and implementing FDA regulations regarding drug/biologic review, approval and distribution; reimbursement strategy and issues; FDA and NIH oversight of clinical trials including registries and reporting of trial results;
Faculty Bios – continued

approval and marketing of orphan drugs; stem cell research and
regulation of cell therapy, gene therapy, tissue engineering and
regenerative medicine products; human subject protection issues
such as IRB review and informed consent, as well as conflicts of
interest and other bioethics issues arising from research and uses
of new technologies.

Before joining Holland & Knight and founding ARM, Michael was
president of The Werner Group, a Washington, D.C.-based firm that
provided lobbying, regulatory, and bioethics consulting services
for biotechnology and pharmaceutical companies, physicians,
health plans, investors, and patient advocacy groups. Prior to
founding The Werner Group, he was chief of policy for the
Biotechnology Industry Organization (BIO), representing over 1000
biotechnology companies in the U.S. and other countries.

Before BIO, he spent six years as counsel for legislation and policy
for the American College of Physicians. Mr. Werner was senior
healthcare advisor to U.S. Senate Majority Leader George Mitchell,
a congressional investigator for the U.S. Senate Special Committee
on Aging and senior advisor to Maryland Governor William Donald
Schaefer.
## Disclosure of Relevant Financial Relationships

<table>
<thead>
<tr>
<th>Name</th>
<th>Disclosure</th>
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<tbody>
<tr>
<td>Jennifer Bernstein</td>
<td>Consulting Fee, Amicus Therapeutics; Consulting Fee, AcelRx Pharmaceuticals; Spouse's Salary, AstraZeneca; Consulting Fee, Prothena Corp</td>
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<tr>
<td>Francesca Cook, MPH</td>
<td>Salary, REGENXBIO</td>
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<td>John Furey</td>
<td>Salary, Spark Therapeutics</td>
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<tr>
<td>Jonathan Garen</td>
<td>Salary and Equity, uniQure</td>
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<tr>
<td>Melissa Hogan, JD</td>
<td>Travel Expenses, Stipends, Shire</td>
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<tr>
<td>Jerry Keybl</td>
<td>Salary, MilliporeSigma</td>
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<td>Laurice Levine, MA, CCLS</td>
<td>Consulting Fee, bluebird bio</td>
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<tr>
<td>Bitao Liang, PhD</td>
<td>Salary, Celgene</td>
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<tr>
<td>Rocio Manghani</td>
<td>Salary and Equity Owner, Kite, a Gilead Company</td>
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<td>Bill Martin</td>
<td>Salary, Express Scripts, Inc.</td>
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<tr>
<td>Lynne Fahey McGrath, MPH, PhD</td>
<td>Salary, Bonus, Stock, REGENXBIO</td>
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<td>Snehal Naik, PhD</td>
<td>Salary and Stock, Johnson &amp; Johnson</td>
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<td>Peter Olagunju</td>
<td>Salary, bluebird bio</td>
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<tr>
<td>Sarah Pitluck, MSc</td>
<td>Salary and Stock, Spark Therapeutics</td>
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<tr>
<td>Robert G. Pietrusko, Pharm.D.</td>
<td>Salary, Voyager Therapeutics, Inc.</td>
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<tr>
<td>Philip R. Reilly, MD, JD</td>
<td>Salary, Third Rock Ventures; Stock, Options, bluebird bio; Stock, Voyager Therapeutics</td>
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<tr>
<td>Mark Trusheim, MSc</td>
<td>Ownership Interest, Co-Bio Consulting</td>
</tr>
<tr>
<td>Kristin Viswanathan Wolff, MPH</td>
<td>Salary, bluebird bio</td>
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Notes