



Alison Bateman-House, PhD, MPH, MA

Alison Bateman-House, PhD, MPH, MA, is an Assistant Professor in the Division of Medical Ethics at NYU Grossman School of Medicine. She is co-chair, with Arthur Caplan, PhD, of the Working Group on Compassionate Use and Preapproval Access, an academic group that studies ethical issues concerning access to investigational medical products and which is composed of patient advocates, clinicians, members of industry, former FDA staffers, lawyers, and academics.

Dr. Bateman-House also co-chairs, with Lesha Shah, MD, the Pediatric Gene Therapy and Medical Ethics (PGTME) working group, which includes academics, patient advocates, industry representatives, and myriad clinical and research professionals. She advises a wide array of biopharmaceutical companies, patient advocacy organizations, and governmental and non-governmental entities about clinical trial design and non-trial access programs, and she serves as an ethicist for three data safety monitoring boards overseeing clinical trials. Dr. Bateman-House also serves as the non-voting, non-paid chair of the NYU/Janssen Pharmaceutical Compassionate Use Advisory Committees (CompACs) for Infectious Diseases and Neurology/Psychology. CompAC won the Reagan-Udall Foundation for the FDA's 2019 Innovation Award.

She has published and spoken extensively on non-trial access to investigational drugs, on clinical trial accessibility, on the history and ethics of using humans as research subjects, and on public health ethics.



Joan Benson, MD, MPH

Dr. Joan Benson leads Merck's strategic engagement with global public health organizations involved in vaccine policies, immunization programs, and funding for vaccines internationally, including WHO, Gavi, UNICEF, and the Bill & Melinda Gates Foundation. She is responsible for establishing and leading key strategic partnerships and programs to address vaccine preventable diseases globally, including in resource limited countries. She has been involved in the development and execution of several partnerships, including numerous HPV and rotavirus vaccination programs with developing country governments and the Merck/Gates Botswana partnerships on HIV/AIDS. A key passion is her tireless effort to advance dialogue and collaborations with diverse partners to tackle cervical cancer and its eventual elimination in developing and emerging countries.

Through partnership with Susan G. Komen for the Cure, she led Merck's participation in the Pink Ribbon Red Ribbon – an initiative to address cervical and breast cancer in sub-Saharan Africa, launched by the George W. Bush Institute, the United States Government through the U.S. Emergency Plan for AIDS Relief (PEPFAR), Susan G. Komen®, and the Joint United Nations Programme on HIV/AIDS (UNAIDS). Dr. Benson served as a public health physician at the Stamford Health Department, and the Southend Medical Center in Stamford, Connecticut, and also was a practicing physician in Nigeria.



Betsy Bogard, MS

Betsy Bogard is dedicated to developing transformative therapies, especially for rare diseases. Her younger brother Jud was born with a rare genetic disease, inspiring her to make a difference for patients and families facing debilitating conditions. Betsy has worked in biotechnology for more than 20 years in areas that include portfolio and alliance management, program leadership, real-world evidence, registries, health economics, and patient community engagement. She is currently the head of program and alliance management for an emerging gene therapy company within the 4:59 Initiative at 5 AM Ventures.

Betsy holds volunteer leadership roles at the International FOP Association, a non-profit patient organization for the rare disease fibrodysplasia ossificans progressiva (FOP), and RARE-X, a non-profit collaborative platform for data sharing in rare diseases. Betsy has a master's degree in health policy and management from the Harvard School of Public Health. She lives in Somerville, Massachusetts with her two sons.



Michael S. Brown, MD

Michael S. Brown received his MD in 1966 from the University of Pennsylvania. He was an intern and resident at the Massachusetts General Hospital and a post doctoral fellow with Earl Stadtman at the National Institutes of Health. He is currently Paul J. Thomas Professor of Molecular Genetics and Director of the Jonsson Center for Molecular Genetics at the University of Texas Southwestern Medical School in Dallas.

Dr. Brown and his colleague, Dr. Joseph L. Goldstein, discovered the low-density lipoprotein (LDL) receptor, which controls cholesterol in blood and in cells. They showed that mutations in this receptor cause Familial Hypercholesterolemia, a disorder that leads to premature heart attacks. Their work laid the groundwork for drugs called statins that block cholesterol synthesis, increase LDL receptors, lower blood cholesterol, and prevent heart attacks. Statins are taken daily by more than 20 million people worldwide.

Brown and Goldstein shared many awards for this work, including the U.S. National Medal of Science and the Nobel Prize for Medicine or Physiology. Dr. Brown served for 16 years on the Board of Directors at Pfizer, and he is currently a Director at Regeneron Pharmaceuticals.



David Cameron

One of the most prominent global influencers of the early 21st Century, David Cameron served as Prime Minister of the United Kingdom from 2010 to 2016, leading Britain's first Coalition Government in nearly 70 years and, at the 2015 General Election, forming the first majority Conservative Government in the UK for over two decades.

As the UK's youngest Prime Minister in two centuries, he came to power at a moment of economic crisis. Under his leadership, the UK's economy was transformed. The deficit was reduced by over two-thirds; one million businesses were set-up, a record number of jobs were created, and Britain became the fastest-growing major advanced economy in the world. That created the stability David needed to cut taxes, introduce a National Living Wage, transform education, reform welfare, protect the National Health Service, and increase pensions.

As part of his domestic agenda, David created Genomics England, who partnered with Illumina to deliver the 100,000 Genomes Project. The project centered around the collection and sequencing of genomes from 100,000 patients with the aim of enhancing research into rare diseases and, as a country with a National Health Service, bring together patient data with genomic sequence information to better understand health and disease. The 100,000th sequence was achieved in December 2018. The program has since expanded to see 1 million whole genomes sequenced by the NHS and UK Biobank over five years from 2018, and there is a further ambitious plan to sequence 5 million genomes over the coming years.

Internationally, David Cameron developed a foreign policy in the post-Iraq era that addressed the new challenges of the Arab Spring, as well as a more aggressive Russia, while ensuring Britain played a full role in the global fight against ISIS. After hosting the successful London 2012 Olympic and Paralympic Games, David chaired the 2013 G8 Summit at Lough Erne in Northern Ireland, where he highlighted the global need for fair taxes, increased transparency, and open trade. He later helped re-write the global goals on aid and sustainable development.



David Cameron, continued

Elected Leader of the Conservative Party in 2005 to reform and modernise a party that had lost three elections in a row, David successfully remodeled the Conservative Party with a new and distinct modern, compassionate agenda – and that is how he governed. He led the way internationally by passing the UK’s Same Sex Marriage Act and offered constitutional referenda on Scotland’s place within the United Kingdom and on the United Kingdom’s place within the European Union.

David continues to focus on issues he advanced while in office: supporting life chances for young people, championing Britain’s cutting-edge medical research, and promoting international development. He is Chairman of Patrons at National Citizen Service, the UK’s flagship youth development programme, President of Alzheimer’s Research UK, and co-chair of the Council on State Fragility, under the auspices of the International Growth Centre. He also serves as an Honorary Governor at The Ditchley Foundation; is a Board member of the ONE Campaign; and together with former US Secretary of State, John Kerry, co-chairs Pew Bertarelli Ocean Ambassadors. Further to these roles, David is advising and working with a number of international businesses, all concentrating on innovative technology-driven sectors, including Fin-Tech, Medi-Tech (Illumina) and AI.

David and his wife, Samantha, have three young children: Nancy, Elwen, and Florence, who was born in 2010 while her father was Prime Minister. Very sadly their much-loved eldest child, Ivan, who suffered from cerebral palsy and Ohtahara Syndrome, a rare and severe form of epilepsy, died in February 2009, aged six.

This experience with his son, Ivan, where David and Samantha were offered such limited genetic information on Ivan’s rare condition, motivated David to pursue this agenda in office, making genomics research a priority in the UK. David hopes that the Genomics England project will lead to the development of personalized treatments that would improve health outcomes worldwide for patients with diseases both common and rare.

David and his family live in London and West Oxfordshire.



Alicyn Campbell, MPH

Alicyn Campbell is currently Head of Digital Health Oncology R&D at AstraZeneca, where she leads on the development of strategies designed to increase their evidence base and improve care through the intelligent use of emerging technologies. Alicyn has over 12 years of experience in Health Outcomes Research. Prior to joining AstraZeneca, she served as the Global Head of Patient Centered Outcomes Research at Genentech/Roche. In that capacity, she was responsible for leadership in the assessment of the patient experience and consulted widely with the FDA and international regulators.

She achieved the first ever novel FDA patient-reported outcome data approved in label for Hycela and was also responsible for novel patient-reported efficacy data for Hemlibra. She is the Founder, Executive Sponsor, and Co-chair of Industry PRO-CTCAE Working Group, recognized as part of the 'Cancer Moonshot' initiative by President Biden, and is a frequent research collaborator to Friends of Cancer Research and LUNGevity. She has also authored several significant scientific publications and presentations, the latest of which was published in *The Lancet Oncology*.



Francis S. Collins, MD, PhD

Francis S. Collins, MD, PhD, was appointed the 16th Director of the National Institutes of Health (NIH) by President Barack Obama. He was sworn in on August 17, 2009. In 2017, President Donald Trump asked Dr. Collins to continue to serve as the NIH Director. President Joe Biden did the same in 2021. Dr. Collins is the only Presidentially appointed NIH Director to serve more than one administration. In this role, Dr. Collins oversees the work of the world's largest supporter of biomedical research, 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 and publication of the entire DNA sequence for a human. He served as director of the National Human Genome Research Institute at NIH from 1993-2008.

Dr. Collins is an elected member of both the National Academy 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. In 2020, he was elected as a Foreign Member of the Royal Society (UK) and was also named the 50th winner of the Templeton Prize, which celebrates scientific and spiritual curiosity.



John F. Crowley

John F. Crowley is the Chairman and CEO of Amicus Therapeutics, a global biotechnology company focused on developing treatments for rare genetic diseases. John has been Amicus CEO since 2005 and has overseen the company's growth from a four-person start-up to one with operations in more than 30 countries, with 500+ employees and a market value of more than \$3 billion. John's involvement with biotechnology stems from the 1998 diagnosis of two of his children with Pompe disease—a severe and often fatal neuromuscular disorder. In his drive to find a cure, he left his position at Bristol-Myers Squibb and became an entrepreneur as the Co-Founder, President, and CEO of Novazyme Pharmaceuticals, a biotech start-up conducting research on a new experimental treatment for Pompe disease (which he credits as ultimately saving his children's lives). In 2001, Novazyme was acquired by Genzyme Corporation and John continued to play a lead role in the development of a drug for Pompe disease as Senior Vice President, Genzyme Therapeutics.

John and his family have been profiled on the front page of *The Wall Street Journal* and are the subjects of a book by Pulitzer prize-winning journalist Geeta Anand, "*The Cure: How a Father Raised \$100 Million-And Bucked the Medical Establishment-In a Quest to Save His Children.*" The major motion picture *Extraordinary Measures*, starring Brendan Fraser and Harrison Ford, is inspired by the Crowley family journey. John is the author of a personal memoir: *Chasing Miracles: The Crowley Family Journey of Strength, Hope, and Joy.*

John served as a commissioned intelligence officer in the U.S. Navy Reserve from 2005-2016, assigned to the United States Special Operations Command, and is a veteran of the global war on terrorism, with service in Afghanistan. He graduated with a BS in Foreign Service from Georgetown University earned a JD from the University of Notre Dame Law School, and completed an MBA from Harvard. The Crowley family was the recipient of the 2011 Family Exemplar Award from the University of Notre Dame. He is also a member of the University Council on Science & Technology at Notre Dame. John served as the National Chairman of the Make-A-Wish Foundation of America (2014-2016) and is a founding Board member of the Global Genes Project. John is a Henry Crown Fellow at the Aspen Institute.





Jonathan A. Epstein, MD

Dr. Epstein graduated from Harvard College in 1983 and Harvard Medical School in 1988, then completed his Residency and Fellowship in Medicine and Cardiology at the Brigham and Women's Hospital, where he also completed an HHMI Postdoctoral Fellowship in Genetics. In 1996 he accepted a position as Assistant Professor of Medicine in the Division of Cardiology at the University of Pennsylvania. From 2006-2015, he served as Chairman of the Department of Cell and Developmental Biology and Scientific Director of the Penn Cardiovascular Institute. He is currently the William Wikoff Smith Professor, Executive Vice Dean, and Chief Scientific Officer at the Perelman School of Medicine at the University of Pennsylvania.

Dr. Epstein has been the recipient of numerous awards, including the Sir William Osler Young Investigator Award from the Interurban Clinical Club (2001), the Outstanding Investigator Award from the American Federation for Medical Research (2006), and the Harriet P. Dustan Award for Science as Related to Medicine from the American College of Physicians (2020). He is a member of the Philadelphia College of Physicians, American Academy of Arts and Sciences, and American Association of Physicians. He is past President of the Interurban Clinical Club and Past President of the American Society for Clinical Investigation. Dr. Epstein is a member of the National Academy of Medicine (previously the Institute of Medicine). He serves on several editorial boards and is a past Deputy Editor of the *Journal of Clinical Investigation*. Dr. Epstein was a founding co-director of the Penn Institute for Regenerative Medicine in 2007.

Dr. Epstein's research has focused on the molecular mechanisms of cardiovascular development to understand and treat human disease. His group has been at the forefront of research, utilizing animal models of congenital heart disease to determine genetic and molecular pathways required for cardiac morphogenesis, which have implications for pediatric and adult cardiovascular disease. Stem cell, angiogenesis, and epigenetic studies have had direct implications for the development of new therapeutic agents for heart failure and myocardial infarction.



David Fajgenbaum, MD, MBA, MSc

David Fajgenbaum, MD, MBA, MSc, is an Assistant Professor of Medicine at the University of Pennsylvania, Associate Director of Patient Impact for the Penn Orphan Disease Center, Founding Director of the Center for Cytokine Storm Treatment & Laboratory, and Executive Director of the Castleman Disease Collaborative Network. He is also the national bestselling author of *Chasing My Cure: A Doctor's Race to Turn Hope Into Action* and a patient battling idiopathic multicentric Castleman disease (iMCD). He is in his longest remission ever thanks to a precision treatment that he identified, which had never been used before for iMCD.

One of the youngest individuals ever appointed to the faculty at Penn Medicine and in the top 1 percent youngest awardees of an NIH R01 grant, Dr. Fajgenbaum leads 18 translational research studies at Penn, including a natural history registry that is meeting an EMA post-approval requirement and a clinical trial of the drug that is saving his life. The innovative approach to research that he has spearheaded through the CDCN—the Collaborative Network approach—has been highlighted as a model for rare disease research in *NEJM*, *Journal of Clinical Investigation*, and *Science*. Now Dr. Fajgenbaum is spreading this approach to other diseases, such as COVID-19.

Dr. Fajgenbaum has been profiled in a cover story by *The New York Times* as well as by Good Morning America, *CNN*, *Forbes* 30 Under 30, and the Today Show. Dr. Fajgenbaum received his BS from Georgetown University, MSc in Public Health from the University of Oxford, MD from the University of Pennsylvania, and MBA from The Wharton School.



Holly Fernandez Lynch, JD, MBE

Holly Fernandez Lynch, JD, MBE, is the John Russell Dickson, MD Presidential Assistant Professor of Medical Ethics in the Department of Medical Ethics and Health Policy at the Perelman School of Medicine. She is also Assistant Faculty Director of Online Education in the Department, helping to lead Penn's first online master's degree (in health care innovation).

Her scholarly work focuses on the ethics and regulation of research with human subjects. As founder and co-chair of the Consortium to Advance Effective Research Ethics Oversight, she leads a collaborative effort to understand, evaluate, and improve IRB quality and effectiveness. Professor Fernandez Lynch was named a Greenwall Faculty Scholar in 2019 to examine the ethics of health care gatekeeping, with an emphasis on access to investigational therapies outside clinical trials (also known as "compassionate use"). In the context of COVID-19, she has applied her expertise to the ethical conduct of research, drug, and vaccine development, and allocation of scarce investigational products during a pandemic.

She served as member of the U.S. Department of Health and Human Services Secretary's Advisory Committee on Human Research Protections from 2014-2019. In 2020, Professor Fernandez Lynch joined the Boards of Public Responsibility in Medicine & Research and the American Society of Law, Medicine, and Ethics. She received her law degree and Master of Bioethics from the University of Pennsylvania.



Pat Furlong

Pat Furlong is the Founding President and CEO of Parent Project Muscular Dystrophy (PPMD), the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy (Duchenne). Their mission is to end Duchenne. They accelerate research, raise their voices in Washington, demand optimal care for all young men, and educate the global community.

Duchenne is the most common fatal, genetic childhood disorder, which affects approximately 1 out of every 3,500 boys each year worldwide. It currently has no cure.

When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, Pat didn't accept "there's no hope and little help" as an answer. Pat immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment and the mechanisms for optimal care. Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne.

In 1994, Pat, together with other parents of young men with Duchenne, founded PPMD to change the course of Duchenne and, ultimately, to find a cure. Today, Pat continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world.



Glen N. Gaulton, PhD

Glen N. Gaulton, PhD, is Vice Dean and Director of the Center for Global Health and Professor of Pathology and Laboratory Medicine at the University of Pennsylvania Perelman School of Medicine. He oversees the full scope of global health activities in the school, with a mission to improve health equity worldwide through improved access to care, discovery and outcomes-based research, and comprehensive educational programs. Prior to this appointment, Dr. Gaulton served for 16 years as the lead scientific officer of the Perelman School. He supervised all aspects of the School's research and research training enterprise, and was responsible for both stimulating new research endeavors and providing the optimal intellectual and administrative support for ongoing research and education programs.

Dr. Gaulton received his PhD in biochemistry and molecular biology from the University of California, Santa Barbara. He conducted postgraduate research in immunology at the School of Public Health and School of Medicine at Harvard University. In 1985 he was appointed Assistant Professor of Pathology and Laboratory Medicine in the School of Medicine at the University of Pennsylvania and in 1998 was appointed full Professor. Dr. Gaulton was appointed Associate Dean and Director of the Combined Degree and Physician Scholar Programs in 1993, Director of Biomedical Graduate Studies in 1995, Vice Dean for Research and Research Training in 1998, and Executive Vice Dean and Chief Scientific Officer in 2006.

Dr. Gaulton's research interests are in the area of viral pathogenesis, early detection of pathogen infection, and outcomes research in global health. He has published over 100 manuscripts and texts and directly supervised the research training of over forty students and fellows. He serves on the Executive Advisory Board of five organizations, is an editor and/or reviewer for nine scholarly journals, and has been chair of four NIH study sections. He has received numerous awards for teaching and research, including the Dean's Award for Basic Science Teaching, the Berwick Memorial Teaching Award, the Lindback Award, the Harry Weaver Neuroscience Scholar Award from the National Multiple Sclerosis Society, and the Leukemia Society Scholar Award.



Jayne Gershkowitz

Jayne joined Amicus Therapeutics in 2006 and serves as Chief Patient Advocate, responsible for developing and executing the global strategies that ensure people living with rare diseases and their families remain at the core of all company operations. A long-time patient advocacy professional, Jayne leads the company's highly regarded Global Patient & Professional Advocacy department, a function that ensures extraordinary patient dedication as the bedrock of Amicus since its earliest days as an R&D organization. Jayne founded the company's Patient Advisory Boards program, which helps give voice to the concerns of patients, families, and caregivers, leads Amicus' Public Policy work to advocate for policies that satisfy unmet needs among those living with rare diseases, and initiated Healing Beyond Disease™, an Amicus initiative to further serve the rare disease community in extraordinary ways. She is a member of the company's Executive Committee, reporting directly to the CEO, and sits on the board of directors.

In February of 2019, Jayne received the second annual "Heart of BioNJ" Award in honor of her selflessness and dedication to patients in the rare disease community and her renowned role as a trailblazer in Patient Advocacy. She is a 2018 PharmaVOICE 100 honoree, recognized for providing inspiration and innovation in the life sciences industry. She is a member of the Boards of Trustees of BioNJ and of the Healthcare Institute of New Jersey and the Corporate Advisory Board of the Child Neurology Foundation. She is a co-founder and vice-chair of the board of Professional Patient Advocates in Life Sciences. Jayne is the former executive director of National Tay-Sachs & Allied Diseases Association.

A native of Medford, Massachusetts, Jayne graduated from Syracuse University with a dual degree in Newspaper Journalism and English Literature and studied marketing management at Radcliffe College. She and her husband, Bruce, reside in Lambertville, NJ; they have two adult children.



Lynn Barghout Jafar

Lynn Barghout Jafar is the co-founder of Loulou Foundation, a private non-profit UK foundation dedicated to advancing research into the understanding and development of therapeutics for CDKL5 deficiency disorder. She is a mother of three who worked in advertising for five years before becoming a full-time mother - serving as primary caregiver for Alia, her eldest daughter who has CDKL5 Deficiency.

Lynn is also the Founder and Managing Mum of her brainchild, High Hopes Pediatric Therapy Center - a place she envisioned in Dubai to enable children with moderate to severe special needs to maximize their potential. Her dream was always to establish a center that offers world-class services through highly experienced therapists in one warm and loving environment.

Lynn holds a BA in Business Marketing from the American University of Beirut and an MSc in Management and Marketing from CASS Business School in London.



Majid Jafar

Majid Jafar works in the oil and gas business as the CEO of Crescent Petroleum which operates in the Middle East, having previously worked for Shell International in Europe. Majid is a supporter of initiatives geared toward improving education and tackling youth employment. He also sits on various non-profit boards including the Board of Fellows of Harvard Medical School, the Academy of the University of Pennsylvania, the Advisory Board of Cambridge University Children's Research Hospital Project, and the Global Precision Medicine Council of the World Economic Forum.

He holds engineering degrees from Cambridge University in England and an MBA from Harvard Business School.



J. Larry Jameson, MD, PhD

J. Larry Jameson, MD, PhD, is Executive Vice President of the University of Pennsylvania for the Health System and Dean of the Raymond and Ruth Perelman School of Medicine. A leading voice in academic medicine nationally, he is the current Chair of the Board of Directors of the Association of American Medical Colleges.

Before joining Penn Medicine in 2011, Dr. Jameson was Dean of the Feinberg School of Medicine and Vice President of Medical Affairs at Northwestern University, positions he held since 2007. He joined Northwestern University Medical School in 1993 as chief of the Division of Endocrinology, Metabolism, and Molecular Medicine. In 2000, he was named Irving S. Cutter Professor of Medicine and Chair of the Department of Medicine.

A prolific physician-scientist and writer, Dr. Jameson has been a pioneer in molecular medicine in the field of endocrinology. His research has focused on the genetic basis of hormonal disorders, and he is the author of more than 350 scientific articles and chapters. Dr. Jameson is Editor-in-Chief of *Harrison's Principles of Internal Medicine*, the most widely used medical text worldwide, and previously served as co-editor of *Jameson and DeGroot's Endocrinology*.

Dr. Jameson received his medical degree with honors and a doctoral degree in biochemistry from the University of North Carolina in 1981. He completed clinical training in internal medicine and endocrinology at Massachusetts General Hospital (MGH) in Boston. Before leaving for Northwestern University, he rose through the ranks at Harvard Medical School to become an Associate Professor of Medicine and chief of the Thyroid Unit at MGH.

Among his many professional distinctions and honors, Dr. Jameson is an elected member of the American Academy of Arts and Sciences and the National Academy of Medicine.





Annalisa Jenkins, MBBS, FRCP

Annalisa Jenkins, MBBS, FRCP is a biopharma thought leader with over 25 years of industry experience. Dr. Jenkins has extensive experience in building and financing biotech companies pursuing cures for the most challenging rare diseases to address important medical issues globally. She has consistently built and led teams advancing programs from scientific research through clinical development, regulatory approval, and into healthcare systems globally. In addition, she is an advocate for diversity and inclusion, particularly for women in science.

Dr. Jenkins served as president and CEO of Dimension Therapeutics, a leading gene therapy company that she took public on the NASDAQ and subsequently sold to Ultragenyx. Prior leadership roles have included the Head of Global Research and Development and Executive Vice President, Global Development and Medical at Merck Serono, and several senior positions at Bristol Myers-Squibb over 15 years - including serving as senior vice president and head of global medical affairs. Earlier in her career, Dr. Jenkins was a medical officer in the British Royal Navy during the Gulf Conflict, achieving the rank of surgeon lieutenant commander.

Dr. Jenkins is a board member of several growing companies, including Oncimmune, AVROBIO, COMPASS Pathways, AOBiome, AgeX, ADOR Diagnostics, MedCity, DMNoMore, Conduit Connect, Affimed, Cocoon Biotech Inc. (Non-Executive Chair), and Kuur Therapeutics (Non-Executive Chair). She also is a committee member of the Science Board to the U.S. Food & Drug Administration, which advises FDA leadership on complex scientific and technical issues, a board member at Faster Cures, a center of The Milken Institute, and Chair of The Court The London School of Hygiene and Tropical Medicine.



Emil Kakkis, MD, PhD

Dr. Kakkis is Chief Executive Officer, President, and Founder of Ultragenyx Pharmaceutical where he leads a team developing and commercializing multiple rare and ultra-rare disease treatments.

Over the last 25 years, Dr. Kakkis is best known for his work developing novel treatments for rare diseases and for advocating on policy issues by founding and supporting the EveryLife Foundation for Rare Diseases. He began his work as an Assistant Professor developing an enzyme replacement therapy (Aldurazyme[®]) for the rare disorder MPS I. After joining BioMarin in 1998, Dr. Kakkis guided the development and approval of two more treatments for rare diseases - Naglazyme for mucopolysaccharidosis (MPS VI) and Kuvan for phenylketonuria (PKU) - and has contributed to the development for four other treatments for rare diseases (Vimizim for MPS IVA, Brineura for CLN2, Palenziq for PKU, and VOSORITIDE for achondroplasia). Dr. Kakkis went on to found Ultragenyx in 2010 to focus on developing more therapeutics for rare and ultra-rare diseases. Since its inception, Ultragenyx has worked on developing treatments for 20 different genetic diseases and has now received four approvals for Crysivita[®] for XLH and TIO, Mepsevii[®] for MPS VII, and Dojolvi for LC-FAOD.

Dr. Kakkis graduated from Pomona College magna cum laude in 1982 with the Vaile Prize for his research, and he received his combined MD and PhD degrees in 1989 from the UCLA (MSTP) with the Emil Bogen Prize for his research. He completed both a Pediatrics residency and Medical Genetics Training Fellowship at Harbor-UCLA Medical Center where he began work on MPS I.

In 2016, Dr. Kakkis received the Roscoe O. Brady Award for Innovation and Accomplishment from the WORLD lysosomal organization and in 2019, he received the Henri A. Termeer Biotechnology Visionary Award from BIO, the biotechnology industry organization.



Luiz Henrique Mandetta, MD

Dr. Luiz Henrique Mandetta holds a bachelor's degree in Medicine from Gama Filho University, Rio de Janeiro (Brazil) and a post-graduation degree in Orthopaedics from the Federal University of Mato Grosso do Sul (Brazil). Dr. Mandetta is also a specialist and fellow in Pediatric Orthopaedics from The Scottish Rite Hospital For Children in Atlanta (USA). Furthermore, Dr. Mandetta holds a Post-Graduation degree in service management and health care from Getúlio Vargas Foundation (Brazil). From January 2019 until April 2020, Dr. Mandetta served as Minister of Health of Brazil.

Dr. Mandetta began his career as a physician at the General Hospital of the Army in Campo Grande (Brazil) and, in 1993, started working at Hospital Santa Casa in Campo Grande (Brazil). From 2001 until 2004, he served as President of Unimed Campo Grande, a health cooperative.

From 2005 to 2010, Dr. Mandetta was the Municipal Secretary of Health of Campo Grande, working intensively to combat one of the most significant infestations of the *Aedes aegypti* mosquito.

From 2011 until 2018, Dr. Mandetta served as federal Congressman representing the state of Mato Grosso do Sul. He was also elected as a Mercosul congressman, from 2011 until 2017. Dr. Mandetta supported initiatives regarding social areas, especially health, social assistance, and education causes. During his second term, Dr. Mandetta was also a member of the Special Commission to monitor actions to combat zika virus.

Since the beginning of 2020, until 2022, former Minister Mandetta will lead the STOP TB Partnership Board, an initiative operated through a secretariat hosted by the United Nations Office for Project Services (UNOPS) in Geneva, Switzerland. His main challenge is to reduce the gap of sustainable investment to fight TB, focusing on the improvement of health systems, digital technologies, and innovation on new diagnostic tests and drugs.



Emma A. Meagher, MD

Emma A. Meagher, MD, serves as Professor of Medicine and Pharmacology at the Perelman School of Medicine at the University of Pennsylvania. Dr. Meagher graduated cum laude with her medical doctorate degree from the Royal College of Surgeons in Dublin, Ireland, and completed postgraduate training in internal medicine, cardiology, and pharmacology.

In her roles as Vice Dean and Chief Clinical Research Officer, and as Senior Associate Vice Provost for Human Research, Dr. Meagher oversees the institution's clinical research infrastructure and its portfolio and sets the strategy for Penn Medicine's clinical research enterprise. Dr. Meagher is also responsible for the rapidly growing portfolio of professional education opportunities provided by the Perelman School of Medicine in her roles as Associate Dean for PSOM Master's and Certificate Programs, and as Director of Translational Research Education.



Dr. Khaled Otaifi

Dr. Otaifi is the General Director of Maternal and Child Health for the Egyptian Ministry of Health. His group is responsible for implementing the national neonatal screening program in Egypt. This program currently screens all neonates in Egypt (2.5 million births annually) for congenital hypothyroidism and PKU, with more than 96% coverage. Dr. Otaifi is working to expand that program to include the main inborn errors of metabolic diseases, especially the diseases that can be tackled through available nutritional measures or specific therapy - while simultaneously preparing to implement national surveillance system for congenital birth defects in Egypt.

As a Consultant of Obstetrics & Gynecology specialized in Assisted Reproduction, Dr. Otaifi is interested in research and new technology that could help prevent and/or treat congenital diseases. In his official position, he is responsible for planning and proposing any updated technology that helps to contribute to the prevention of any form of disability as one of the priorities of the Egyptian Ministry of Health and Population. Dr. Otaifi has a dream to establish an outstanding National Genetic Institute in Egypt, including facilities for research and intervention for new modalities in genetic science. He believes that this can be achieved through national and international cooperation.



Daniel J. Rader, MD

Daniel Rader, MD, is the Seymour Gray Professor of Molecular Medicine and Chair of the Department of Genetics at the Perelman School of Medicine at the University of Pennsylvania. He also serves as Chief of the Divisions of Human Genetics in the Departments of Medicine and Pediatrics. Dr. Rader is Associate Director of Penn's Institute for Translational Medicine and Therapeutics and directs the Penn Medicine BioBank, an integrated resource to support human genetics and translational research.

Dr. Rader trained in internal medicine at the Yale-New Haven Hospital and in human genetics and physiology of lipid metabolism at the National Institutes of Health. He was recruited to Penn in 1994.

Dr. Rader's research focuses on the human genetics and functional genomics of lipid metabolism and atherosclerosis, as well as the translational implications for novel therapeutic approaches. He 'rescued' an abandoned molecule that reduces cholesterol and led the successful development of this compound as a novel therapeutic for patients with homozygous familial hypercholesterolemia, a genetic form of severely elevated cholesterol. He is known for his work in the metabolism and function of HDL (the 'good cholesterol'). He has led pioneering studies of novel biological pathways in lipid metabolism and heart disease discovered through genome-wide human genetics studies. He has been an international leader in the translation of human genetics into novel therapeutic targets. He has been a champion of 'genomic medicine' and its potential benefits in guiding preventive therapies. He has been involved in several start-up biotech companies related to his work.

Dr. Rader sees patients with lipid disorders and has been regularly recognized as one of America's Top Doctors and Best Doctors in America. He is a recipient of several national and international awards for his research contributions. He has been elected to the National Academy of Medicine and the American Academy of Arts and Sciences.





Jeremy Snyder, PhD

Jeremy Snyder is a Professor in the Faculty of Health Sciences at Simon Fraser University in British Columbia, Canada. He received his PhD in Philosophy from Georgetown University. Dr. Snyder's research interests focus on ethical issues arising from international trade, especially trade in health services. He is currently engaged in research projects on medical crowdfunding, medical tourism, and the exploitation of hope for a better life. His book *Exploiting Hope: How the Promise of New Medical Interventions Sustains Us - and Makes Us Vulnerable* was published by Oxford University Press in 2020.



George Weiss

George Weiss is the Chief Executive Officer of Weiss Multi-Strategy Advisers, LLC, an investment management firm with a 42 year history. He earned a bachelor's degree from the University of Pennsylvania's Wharton School of Finance in 1965. Mr. Weiss is the founder of the Weiss Family Foundation, a private foundation dedicated to improving lives through education and healthcare.

Mr. Weiss started the Orphan Disease Pathway Project in 2008 to find cures and effective treatments for rare diseases. Partnering with the Orphan Disease Center at Penn, the organization's mission is to facilitate and expedite the development of novel therapies and the translation of these therapies into the clinic.

Mr. Weiss is a former Trustee and member of the Executive Committee at Penn Medicine. He has worked extensively with the Abramson Cancer Center and raised significant money for Dr. Carl June's CAR-T cell research. Mr. Weiss is the former Vice Chair of the Board of Trustees of the University of Pennsylvania. In 2014 Mr. Weiss received an honorary Doctor of Laws from the University of Pennsylvania. He has also received honorary degrees from Syracuse University, Lesley College, Trinity College, Colby-Sawyer College and St. Joseph's College-Maine.

Mr. Weiss is the founder and chairman of Say Yes to Education, Inc., a national non-profit organization committed to opening access to post-secondary education for high-poverty youth. He created Say Yes to Education in 1987 with a promise to pay the full costs of college or vocational training for 112 students in Philadelphia; since then, the Say Yes program has grown to include over 170,000 students in eight cities.

Mr. Weiss is a seventh-degree Black Belt in KiToshaKai and Tae Kwon Do. He was a Member of the United States Martial Arts team from 2002-2006.





James M. Wilson, MD, PhD

James M. Wilson, MD, PhD, is a Professor of Medicine in the Perelman School of Medicine at the University of Pennsylvania, as well as Director of the Orphan Disease Center and Director of the Gene Therapy Program. Dr. Wilson began his work in gene therapy during his graduate studies at the University of Michigan nearly 40 years ago. He then moved to Boston to do a residency in Internal Medicine at the Massachusetts General Hospital and continued his work in gene therapy at MIT. Dr. Wilson has been at the nexus of this emerging therapeutic area from its birth. He created the first and largest academic-based program in gene therapy after being recruited to Penn in 1993. He initially focused on the clinical translation of existing gene transfer technologies but soon redirected his efforts to the development of second and third generation gene transfer platforms.

His laboratory discovered a family of viruses from primates called adeno-associated viruses (AAV) that could be engineered to be very effective gene transfer vehicles. These so called "vectors" have become the technology platform of choice and have set the stage for the recent resurgence of the field of gene therapy. Dr. Wilson has also been active in facilitating the commercial development of these new gene therapy platforms through the establishment of several biotechnology companies. He is currently leading a national dialogue on the challenges of commercializing these potentially lifesaving treatments due to the disruptive nature they will have on traditional business models. Throughout his career, the focus of Dr. Wilson's research has been rare inherited diseases, ranging from cystic fibrosis to dyslipidemias to a variety of neurologic disorders.

Dr. Wilson has published over 580 papers, reviews, commentaries, and editorials in the peer-reviewed literature and is an inventor on over 153 U.S. patents and patent applications and over 200 total issued patents worldwide. He was the second President of the American Society of Gene Therapy. Dr. Wilson was the 2014 recipient of the William Osler Patient Oriented Research Award of the University of Pennsylvania and received the 2015 Scientific Achievement Award from Pennsylvania Bio. Dr. Wilson was noted by the journal *Nature Biotechnology* to be the "second most productive bio-entrepreneur in life sciences."





Janet Woodcock, MD

Janet Woodcock was named Acting Commissioner of Food and Drugs on January 20, 2021.

As Acting Commissioner, Dr. Woodcock oversees the full breadth of the FDA portfolio and execution of the Federal Food, Drug, and Cosmetic Act and other applicable laws. This includes assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines, and other biological products for human use, and medical devices; the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation; and the regulation of tobacco products. Dr. Woodcock began her FDA career in 1984, joining the agency's Center for Biologics Evaluation and Research (CBER) as Director of the Division of Biological Investigational New Drugs, as well as serving as CBER's Acting Deputy Director for a period of time. She later became Director of the Office of Therapeutics Research and Review in CBER, which included the approval of the first biotechnology-based treatments for multiple sclerosis and cystic fibrosis during her tenure.

In 1994, Dr. Woodcock was named Director of the FDA's Center for Drug Evaluation and Research (CDER), overseeing the center's work that is the world's gold standard for drug approval and safety. There she led many of the FDA's drug initiatives, including introducing the concept of risk management as a new approach to drug safety; modernizing drug manufacturing and regulation through the Pharmaceutical Quality for the 21st Century Initiative; advancing medical discoveries from the laboratory to consumers more efficiently under the Critical Path Initiative, and launching the Safety First and Safe Use initiatives designed to improve drug safety management within and outside the FDA, respectively.

In 2004, Dr. Woodcock became Deputy Commissioner and Chief Medical Officer in the Office of the Commissioner. Later she took on other executive leadership positions in the Commissioner's Office, including Deputy Commissioner for Operations and Chief Operating Officer.



Janet Woodcock, MD, continued

In 2007, Dr. Woodcock returned as Director of CDER until she was asked to lend her expertise to “Operation Warp Speed” for developing therapeutics during the COVID-19 pandemic, such as evaluating the potential benefits of monoclonal antibody treatments for certain COVID-19 patients.

From late 2020, she split her time advising “Operation Warp Speed” on advancing COVID-19 therapeutics while also serving as the Principal Medical Advisor to the Commissioner on key priorities on behalf of the Office of the Commissioner. Dr. Woodcock holds a Bachelor of Science in chemistry from Bucknell University (Lewisburg, PA), and a Doctor of Medicine from the Feinberg School of Medicine at Northwestern University Medical School (Chicago). She also completed further training and a fellowship in rheumatology, as well as held teaching appointments at the Pennsylvania State University and the University of California in San Francisco. She is board certified in internal medicine.

Dr. Woodcock has been bestowed numerous honors over her distinguished public health career, most notably: a Lifetime Achievement Award in 2015 from the Institute for Safe Medication Practices; the Ellen V. Sigal Advocacy Leadership Award in 2016 from Friends of Cancer Research, the Florence Kelley Consumer Leadership Award in 2017 from the National Consumers League, and the 2019 Biotechnology Heritage Award from the Biotechnology Innovation Organization and Science History Institute.



Tachi Yamada, MD

Dr. Tadataka (Tachi) Yamada is a Venture Partner with Frazier Healthcare Partners. As a member of the Frazier Life Sciences team, he focuses on creating companies as well as providing strategic guidance to existing portfolio companies. He chairs the boards of Phathom Pharmaceuticals and Passage Bio and serves as Director for a number of private life science companies. Prior to joining Frazier, Dr. Yamada was Executive Vice-President, Chief Medical & Scientific Officer, and a Board Member of Takeda Pharmaceuticals. Dr. Yamada has also served as President of the Bill & Melinda Gates Foundation Global Health Program. He was formerly Chairman, Research and Development, and a Member of the Board of Directors of GlaxoSmithKline, and before that he was Chair of the Department of Internal Medicine and Physician-in-Chief at the University of Michigan Medical Center.

Dr. Yamada holds a bachelor's degree in history from Stanford University and obtained his MD from New York University School of Medicine. In recognition of his contributions to medicine and science, he has been elected to membership in the National Academy of Medicine (US), the American Academy of Arts and Sciences, the Academy of Medical Sciences (UK), and the National Academy of Medicine (Mexico). He has been conferred an honorary appointment as Knight Commander of the Most Excellent Order of the British Empire (KBE), the Honorary Citizen Award from the Government of Singapore, and the Order of the Rising Sun, Gold and Silver Stars from the Japanese Government. He is a Past-President of the Association of American Physicians and of the American Gastroenterological Association, and he has served as a member of the President's Council of Advisors on Science and Technology and the Advisory Committee to the Director of the NIH. He was formerly Vice Chair of the Council of the National Academy of Medicine and currently serves as Chairman of the Board of Directors of the Clinton Health Access Initiative.



Timothy Yu, MD, PhD

Dr. Tim Yu is a neurologist and researcher at Boston Children's Hospital. He completed his MD and PhD at UC San Francisco and neurology residency at Massachusetts General Hospital and Brigham and Women's Hospital. Currently, he is Associate Professor at Harvard Medical School and Associate Member of the Broad Institute of MIT and Harvard. His research group in the Division of Genetics and Genomics works at the intersection of genetics, neurobiology, and bioinformatics to understand the basis for brain disease and advance genomic medicine. His current projects include computational genomic analyses of large autism cohorts to identify disease genes, genome sequencing for newborn screening and neonatal ICU care, and the development of platform approaches for individualized therapy of rare neurogenetic diseases.