**Chair: Arthur L. Caplan**, PhD, is the Drs. William F. and Virginia Connolly Mitty Professor and founding head of the Division of Medical Ethics at NYU Langone Health. He is the chair of the Compassionate Use Advisory Committees (CompAC) and the author or editor of 32 books and more than 700 papers in peer-reviewed journals. His most recent book is *Vaccination Ethics and Policy*, with Jason Schwartz (MIT Press, 2017).

**Co-chair: Alison Bateman-House**, PhD, MPH, MA, is an assistant professor in the Division of Medical Ethics at NYU Langone Health’s School of Medicine. She is co-chair of the Working Group on Compassionate Use and Preapproval Access (CUPA), an academic group that studies ethical issues concerning access to investigational medical products and is composed of patient advocates, clinicians, members of industry, former FDA staffers, lawyers, and academics. Bateman-House also serves as the non-voting, non-paid chair of the NYU/Janssen Pharmaceutical Compassionate Use Advisory Committees (CompAC) for Infectious Diseases and Neurology/Psychology. Individually, with CUPA, and with CompAC, she has published and spoken extensively on how to best handle requests for non-trial access to investigational drugs and on related ethical issues. She has also written and spoken frequently on the history and ethics of using humans as research subjects and on clinical trial accessibility.

**Jinsy A. Andrews**, MD, MSc, is an assistant professor of neurology in the Division of Neuromuscular Medicine at Columbia University and serves as the director of neuromuscular clinical trials in the Department of Neurology. Prior to this position, she helped develop and direct a clinical trials unit for neurological diseases at the Hospital for Special Care/University of Connecticut. More recently she was the head of neuromuscular therapeutics at Cytokinetics, where she focused on developing investigational therapies for neuromuscular diseases. She has extensive experience in conducting human clinical trials in neuromuscular disorders from Phase 1 to Phase 3 in both academic and industry settings, as well as participating in expanded access programs. She received her BS from Union College, her MSc in Biostatistics (Patient-Oriented Research) from Columbia University’s Mailman School of Public Health, and her MD from Albany Medical College. She completed her residency in neurology at the University of Connecticut and completed fellowship training in both neuromuscular medicine and clinical neurophysiology at Columbia. She is board certified in neurology, neuromuscular disease, and electrodiagnostic medicine.

**Nancy Beck**, PhD, has worked in regulatory science and health policy for the last 11 years, focusing on advancing new tools and approaches for solving complex, systemic challenges in drug development and approval. Most recently, she was a director at Avalere Health, providing regulatory and policy analysis on issues across the drug development landscape. She advised clients on strategies for product development and
lifecycle management, patient engagement, pre-approval access, real world evidence, innovative clinical trial designs, and novel clinical endpoints. She held several positions at the Reagan-Udall Foundation for the FDA (RUF), a nonprofit created by Congress to support the FDA’s mission through the development of multisector, multidisciplinary public-private partnerships. She built the legal, scientific, and operational structures for several partnerships during at RUF, including the Expanded Access Navigator, a website that provides physicians and patients with information on the pre-approval access process. Prior to RUF, she worked as a science and policy adviser, specializing in development and adoption of more efficient and predictive approaches to safety assessment spanning the pharmaceutical, cosmetic, pesticide, and industrial chemical sectors. She received her doctorate in microbiology and immunology from the University of Michigan and conducted post-doctoral research at Dartmouth Medical School.

**Hayley M. Belli**, PhD, MS, has been a postdoctoral fellow in the Division of Biostatistics in the Department of Population Health since December 2017. She conducts research in the design, implementation, and statistical analysis of clinical trials with a focus on adaptive, pragmatic studies. Her work is supported through the New York University Clinical and Translational Science Institute TL1 Scholar Program. Prior to NYU, Hayley completed her PhD in biomedical engineering at Northwestern University. Her thesis focused on neural engineering, specifically tactile perception and developing models for comparing geometric and mechanical properties of whisker arrays across active sensing species. While at Northwestern she was supported for three years through an NIH F31 National Research Service Award; taught courses in biomedical statistics, linear algebra, systems neuroscience, and fluid mechanics; and completed an MS in applied mathematics. She is thrilled to bring her interdisciplinary knowledge to the Working Group on Compassionate Use and Pre-Approval Access.

**Carolyn Riley Chapman**, PhD, MS, has been a postdoctoral fellow in the Division of Medical Ethics since September 2017. She has enjoyed a wide range of academic, professional, and volunteer experiences. She was interim associate director of the Columbia University bioethics program from February through June 2016 and associate/lecturer for the program from July 2016 through August 2017. She has worked as a freelance science/medical writer and has published articles in MedPage Today, Voices in Bioethics, Start-Up, Drug Discovery & Development, and Genetic Engineering News. She worked at L.E.K. Consulting as a business strategy consultant in the biotech/pharma industry and contributed to the growth of a biopharmaceutical company from its earliest stages, as the third employee at Aton Pharma, a start-up that developed a cancer drug discovered in Columbia University and Memorial Sloan Kettering laboratories. After graduating from Dartmouth College summa cum laude with high honors in biology, she earned a PhD in genetics from Harvard University, where she studied studying cell cycle checkpoints in the fission yeast *S. pombe*. She also has an MS in bioethics from Columbia University.

**David R. Curry**, MS, is a co-founder and president of the GE2P2 Global Foundation, a U.S.-based NGO with the mission to advance ethical and scientific rigor in research and evidence generation across health, human rights, humanitarian response, and
development. The foundation’s Center for Access to Medicines focuses on bioethical issues associated with research, discovery, and clinical trials for investigational medicines; compassionate use/pre-licensure/expanded access programs, and access to essential medicines in low resource settings and humanitarian contexts globally. Since 2008 he has served as executive director of the Center for Vaccine Ethics and Policy (CVEP), a foundation program that focuses on ethical and policy issues associated with the full vaccine life cycle, with a special interest in vaccines and immunization in LMIC/Global South contexts, as well as in disaster, conflict, humanitarian, and emergency settings. He holds an appointment as associate faculty of the Division of Medical Ethics.

Nancy Dubler, LLB, is Consultant for Ethics for the New York City Health and Hospitals Corporation, an adjunct professor at the Division of Medical Ethics at NYU Langone Health, professor emerita of bioethics at the Albert Einstein College of Medicine, and the founding director of the Montefiore Medical Center Division of Bioethics and the Montefiore/Einstein Certificate Program in Bioethics and Medical Humanities.

Kelly McBride Folkers, MA, is a research associate at the Division of Medical Ethics at NYU Langone Health and an adjunct lecturer at the City University of New York. Her work focuses on a variety of topics related to pre-approval access to investigational medical products including the role of IRBs in expanded access, clinical trial equity, medical crowdfunding, innovative treatment for transgender youth, use of real-world evidence in regulatory decision-making, and federal expanded access policy. She participates in several Compassionate Use Advisory Committees (CompAC) and runs the Division’s High School Bioethics Project, a freely available online curriculum for high-school science classes that provides resources for teacher training and student education. Before pursuing a career in bioethics, she worked in basic cancer research at the National Cancer Institute, Uppsala University, and Memorial Sloan Kettering Cancer Center. She has an MA in bioethics from NYU and a BS in biology from Denison University.

Pat Furlong, MS, RN, BSN, is considered one of the foremost authorities on Duchenne muscular dystrophy in the world. She is the founding president and CEO of Parent Project Muscular Dystrophy (PPMD), the largest nonprofit organization in the U.S. focused solely on Duchenne, the most common fatal, genetic childhood disorder. It affects 1 in 4,600 boys worldwide and has no cure. When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, she immersed herself in research, working to understand the pathology of the disorder, the extent of research investment, and the mechanisms for optimal care. In 1994, together with other parents of young men with Duchenne, she founded PPMD to change the course of the disease and, ultimately, find a cure. PPMD’s 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.

Claudia Hirawat is the executive chair of MK&A, a firm that supports the development of biopharmaceutical products by enhancing the relationships among stakeholders such as
patient groups, regulators, and disease experts. She acquired MK&A in May 2018. She is an adviser to biopharmaceutical companies on business development and corporate strategy and is a volunteer for EURORDIS, a nonprofit alliance of more than 700 rare disease patient organizations. She was chair of the International Circle of Ambassadors for EURORDIS for three years, on the advisory board of the National Hemophilia Foundation, and on the board of directors of Parent Project Muscular Dystrophy. Until January 2015 she was president of PTC Therapeutics, Inc., where she led corporate and business development as well as multiple rounds of financing, including the company’s pivotal mezzanine financing and IPO. Before that, as senior vice president of corporate development, she spearheaded PTC’s multiple collaborations (including transactions with Genzyme, Pfizer, Schering-Plough, Hoffman-La Roche, and Celgene), which generated more than $300 million in cash in addition to milestones and royalties. Before joining PTC, in 2000, she was vice president of LedbetterStevens, a management consulting firm for the biopharmaceutical industry.

Kay Holcombe, MS, serves as senior adviser to the Milken Institute, focusing on the Lynda and Stewart Resnick Center for Public Health. She recently retired as senior vice president for science policy at BIO (Biotechnology Innovation Organization). Before that, she was vice president for government relations at Sanofi-Genzyme; executive vice president of Policy Directions Inc.; professional health legislative staff and senior health policy adviser for the House of Representatives Energy and Commerce Committee; professional health legislative staff for the Senate Labor and Human Resources Committee; deputy associate commissioner for legislative affairs, FDA; executive vice president of the Foundation for Biomedical Research; associate director for public health legislation, HHS Office of the Assistant Secretary for Legislation; deputy associate administrator for planning, evaluation, and legislation, Health Resources and Services Administration; special assistant to the director, Division of Legislative Affairs, NIH; executive secretary, National Heart, Lung, and Blood Institute National Advisory Council. She is on the boards of the Reagan-Udall Foundation for the FDA, the National Blood Clot Alliance, and the Critical Path Institute. She received an MS in chemistry, graduating with honors, from the University of Virginia and was elected to Phi Beta Kappa, Phi Kappa Phi, and Iota Sigma Pi.

Lisa Kearns, MS, MA, is a senior research associate at the Division of Medical Ethics. She has spoken and published extensively on state and federal “right to try” legislation and has served as an expert on the subject for two external organizations. She is the deputy chair of the Compassionate Use Advisory Committees (CompAC) Oncology and Neurology/Psychology panels. She has an MA in philosophy from UNC Chapel Hill and an MS in bioethics from Columbia.

Laura Kimberly, MSW, MBE, is an assistant research scientist in the Hansjörg Wyss Department of Plastic Surgery and an associate of the Division of Medical Ethics at NYU Langone Health. She is also a doctoral candidate in social policy and administration at Columbia University’s School of Social Work. Her research focuses on organ transplantation, aging, and health, with particular attention on the ethical and psychosocial dimensions of health and illness. She has worked previously in population
health research and education and in social policy administration. She is interested in social justice considerations, including equitable access to health care services. She holds a BA in philosophy from Yale University and master’s degrees in social work and bioethics from the University of Pennsylvania.

**Richard Klein**, an internationally recognized expert in pre-approval access to therapeutic agents, worked at the FDA for more than 40 years, before leaving the agency in 2017. While there he helped develop the revised expanded access regulations and guidelines, led the creation of the FDA expanded access website, and helped develop the streamlined application for individual patient access and the Expanded Access Navigator. He also spearheaded the effort to waive full-board IRB review for individual patient access to unapproved drugs and biologics. As director of the FDA’s Patient Liaison Program, he interacted extensively with outside communities and the agency’s scientific and policy offices to advocate for patient interests and facilitate patient engagement in regulatory activities. He worked closely with patient communities in a variety of areas, including treatment access to unapproved drugs, product safety, and clinical trial design. Before taking on that role, he created the FDA’s HIV/AIDS program, working with AIDS activists and advocates to coordinate their input and participation in regulatory policy and decision-making related to HIV/AIDS. Prior to working in patient engagement, he helped develop policies and regulations for the protection of human research subjects and provided guidance for institutional review boards (IRBs).

**Andrew McFadyen**, BA, B.Ed, is the executive director for the Isaac Foundation, a non-profit organization he founded to fund innovative research projects that aim to find a cure for MPS, a rare and progressive disease affecting his eldest son. He has led numerous advocacy efforts helped to shape public policy throughout Canada with respect to availability of treatments for children dying from rare diseases. In 2013, he created a second non-profit corporation, called Equal Access for Rare Disorders, and has continued work fighting for fair and equitable access to treatments for children affected by rare diseases throughout Canada and the United States. In addition to working with families, he visits classrooms and delivers presentations to pharmaceutical companies about his experiences working with families and government. He lives in Campbellford, Ontario with his wife Ellen, and their two children, Isaac and Gabriel.

**Lindsay McNair**, MD, MPH, MSBioethics, is the chief medical officer for the WIRB-Copernicus Group. Before joining WCG, she was a consultant to multiple biopharma companies, providing medical guidance on clinical development strategies, study designs for new drug studies, and medical oversight of all phases of clinical trials. Prior to that, she was the medical lead for the telaprevir development program at Vertex Pharmaceuticals. She is adjunct faculty at Boston University and teaches graduate courses on the scientific design of clinical research studies. She now oversees the physician team within the WCG IRBs, and provides consultation to institutions and companies on protocol design, regulatory compliance, human subjects protection, and ethical policy development. She graduated from the University of Connecticut School of Medicine and trained in general surgery at Boston University Medical Center. She earned an MPH from Boston University and an MS in bioethics from Union Graduate College.
She was a member of the Boston University Medical Center IRB and a site visitor for the Association of the Accreditation of Human Research Protection Programs (AAHRPP). She serves on the Multi-Regional Clinical Trials Center committee at Harvard and is an associate editor of the Journal of Empirical Research on Human Research Ethics.

Jennifer Miller, PhD, is an assistant professor at Yale University School of Medicine as well as founder of the New York–based nonprofit Bioethics International and the Good Pharma Scorecard. She is also a member of the World Economic Forum. Previously, she was based at NYU School of Medicine, Duke University, and Harvard University. She served on NYU’s Pharmacy and Therapeutics Committee, the CDC Task Force for Pediatric Emergency Mass Critical Care, the AMA Advanced Disaster Life Support Education Consortium, the UN Economic and Social Council as a consultant, and the PCORI-NIH Collaboratory. Her work has appeared in 47 publications, including Nature Medicine, Health Affairs, JAMA Internal Medicine, Hastings Center Report, and Clinical Trials.

Kenneth I. Moch, president and CEO of Cognition Therapeutics, a developer of novel medicines for Alzheimer’s disease, has broad experience building, financing, and leading private and public life science companies. In addition to Cognition, Ken has been the co-founder or CEO of four companies that have pioneered novel therapies for life-threatening diseases: Chimerix (antiviral), Alteon (diabetes and cardiovascular), Biocyte (cord blood stem cell transplantation), and the Liposome Company (anti-cancer and antifungal). He has been a managing partner of the Salutramed Group, CEO of BioMedical Enterprises, managing director of Healthcare Investment Banking at ThinkEquity Partners, and a management consultant with McKinsey & Company. He has served for more than a decade on the board of the Biotechnology Innovation Organization (BIO), where he currently chairs BIO’s Bioethics Committee and co-chairs the Emerging Company Section’s Policy Subcommittee, and is a past chairman of BioNJ. He is on the boards of Zynerba Pharmaceuticals and Gamida Cell and served for more than a decade on the Board of M2Gen, the personalized medicine subsidiary of the Moffitt Cancer Center. He holds an AB in biochemistry with a minor in health policy from Princeton University and an MBA from the Stanford University Graduate School of Business.

Robert “Skip” Nelson, MD, M.Div, PhD, is senior director, Pediatric Drug Development, in the Child Health Innovation Leadership Department (CHILD) at Johnson & Johnson. From 2006 to 2017 he was the deputy director and senior pediatric ethicist in the Office of Pediatric Therapeutics at FDA, providing consultation throughout the agency on ethical issues arising in the development of FDA-regulated products for children, and serving as a standing member of the internal FDA Pediatric Review Committee. Prior to joining FDA full-time in 2009, he was a professor of anesthesiology, critical care, and pediatrics at the Children’s Hospital of Philadelphia and University of Pennsylvania School of Medicine. After receiving an MD from Yale University, he trained in pediatrics (Massachusetts General Hospital) and neonatology and pediatric critical care (University of California, San Francisco), and remains board certified in all
three areas. He has a Master of Divinity degree from Yale Divinity School and a PhD in the Study of Religion from Harvard University, specializing in ethics.

**Barbara Redman**, RN, PhD, MBE, is an associate of the Division of Medical Ethics. Her work focuses on research ethics and to a lesser extent chronic disease ethics. In the Working Group, she has focused on the role of IRBs in approving pre-approval access, publishing (with Alison Bateman-House) “Institutional Review Boards as Arbiters of Expanded Access to Unapproved Drugs: Time for a Change?,” *Therapeutic Innovation & Regulatory Science*, epub ahead of print.

**Christopher Robertson**, JD, PhD, was a visiting professor at NYU Law in 2016–2017, on leave from University of Arizona, where he is associate dean for Research and Innovation and founder of the Regulatory Science Program. The editor of two books, author of another, and with more than 50 journal articles to his name, he is an expert in health law and the intersection of law and science. He graduated magna cum laude from Harvard Law School, where he also served as a Petrie Flom fellow and, later, visiting professor. He earned a PhD in philosophy at Washington University in St. Louis, where he also taught bioethics.

**David I. Scheer**, MS, is president of Scheer & Company, Inc., a venture capital, corporate strategy, and transactional advisory services firm focused on the life sciences. He has been involved in the founding and/or been a board member of many life sciences and biotechnology companies. He is a founder and board member of Achillion Pharmaceuticals, where he served as board chair for eight years, and was affiliated with the health care investing team at Oak Investment Partners. He has worked with the Harvard School of Public Health, the Rutgers Global Health Institute, and the National Organization for Rare Disorders (NORD) and is on the executive board of the Center for Biomedical and Interventional Technology at Yale. He has been a guest lecturer and/or panelist at the Wharton School of the University of Pennsylvania, the University of New Haven, and the Yale School of Management, and an executive-in-residence at the Carey School of Business at Johns Hopkins. He is a senior adviser on research commercialization at SickKids Hospital and at the University Health Networks in Toronto. He received an AB, cum laude, in biochemical sciences from Harvard College and an MS in cell, molecular, and developmental biology from Yale University.

**Lesha D. Shah**, MD, is a clinical assistant professor of Child and Adolescent Psychiatry at the NYU School of Medicine. She received an undergraduate degree from Barnard College, Columbia University. After receiving her medical degree, she completed her psychiatry residency at Tufts University and a fellowship in child and adolescent psychiatry at the Icahn School of Medicine at Mount Sinai, and is dually board-certified. She has led an inpatient adolescent psychiatric unit and currently provides psychiatric consultation in the acute medical and surgical setting as well as in pediatric primary care. She studies issues of consent and capacity as they interface with family complexity, psychiatric illness, and innovative medicine. She also teaches undergraduate and medical students, residents, and fellows and is interested in physician perspectives around medical
decision-making. She is a fellow of the American Psychiatric Association and has served on the APA Council on Psychiatry and the Law.

J. Russell Teagarden, DMH, MA, has worked in acute care provider institutions, scientific communications, pharmacy benefit management, and patient advocacy. His roles have included: clinical pharmacy practice; clinical research; health services research; institutional drug policy development; prescription drug coverage policy development and clinical management; health professions education and training; and executive management. He has a BS in pharmacy from the University of Illinois College of Pharmacy, an MA in research methodology from Loyola University of Chicago, and a doctor of medical humanities degree from Drew University. He completed a residency in hospital pharmacy at Northwestern University Medical Center and was a visiting scholar at the NIH’s Bioethics Department. He has served as a trustee for nonprofit health care organizations and as a member of advisory panels for several government and quasi-government organizations.

David Wallach, MPH, CIP, joined NYU in May 2016 as director of Research Regulatory Services. He oversees the human research quality assurance and quality improvement program, ClinicalTrials.gov registration and reporting, and regulatory services, providing support for faculty-held INDs and IDEs. For more than 14 years he worked at the Albert Einstein College of Medicine IRB, culminating in his role as the director of the IRB, where he oversaw IRB operations for Albert Einstein College of Medicine, Montefiore Medical Center, Jacobi Medical Center, North Central Bronx Hospital, and Yeshiva University.

Tom Watson, BSc, executive vice president, early access programs, Bionical Group, has for the last five years partnered with pharma and biotech companies to design strategies for pre-approval access and develop global programs, allowing patients to gain access to treatments that would otherwise be unavailable — all while helping to set up and run more than 200 global programs. Previous roles include executive director for TW Consulting Group and head of U.S. business development for IDIS Managed Access, part of the Clinigen Group. He has developed thinking and strategy for many of the top 20 pharma companies in this important area. Before working in pre-approval access, he spent 14 years in the pharmaceutical industry in a variety of leadership roles, including head of marketing for a multinational pharma company. During this time he also led a wide range of global pre-launch and launch activities for treatments addressing areas of high unmet medical need.