

Amyotrophic Lateral Sclerosis: Accelerating Treatments and Improving Quality of Life

Committee

Alan I. Leshner

Chair

Alan I. Leshner (Chair) is Chief Executive Officer, Emeritus of the American Association for the Advancement of Science (AAAS) and former Executive Publisher of the journal Science. Before joining AAAS, Dr. Leshner was Director of the National Institute on Drug Abuse at the National Institutes of Health. He also served as Deputy Director and Acting Director of the National Institute of Mental Health, and in several roles at the National Science Foundation. Previously, Dr. Leshner was Professor of Psychology at Bucknell University. Dr. Leshner is an elected fellow of AAAS, the American Academy of Arts and Sciences, and many others. He is a member and served as Vice Chair of the governing Council of the National Academy of Medicine of the National Academies of Sciences, Engineering and Medicine. He served two terms on the National Science Board, appointed first by President Bush and then reappointed by President Obama. Dr. Leshner received M.S. and Ph.D. degrees in physiological psychology from Rutgers University and an A.B. in psychology from Franklin and Marshall College. Dr. Leshner has received many honors and awards, including the Walsh McDermott Medal from the National Academy of Medicine and seven honorary Doctor of Science degrees.

Suma Babu

Member

Suma Babu is an Assistant Professor of Neurology at Harvard Medical School, co-director of the Neurological Clinical Research Institute at Massachusetts General Hospital (MGH) and provides clinical care for people with ALS and other motor neuron diseases at the multidisciplinary ALS clinic at the MGH Sean M. Healey and AMG Center for ALS. She has a special interest in developing disease modifying treatments and clinical trial biomarker readouts for people with motor neuron diseases. Her clinical research work is primarily aimed at improving ALS patient care and survival outcomes. As the overall principal investigator, she has led two multisite early-phase biomarker-driven clinical trials in ALS (MN-166 in ALS- investigator initiated, LAM-002A in C9orf72 ALS- protocol PI for OrphAI therapeutics industry-sponsored trial). She is a multiple principal investigator on several NIH-U01 expanded access protocols (EAPs) including the awarded Trehalose 25-site EAP (Seelos), Pridopidine 45 site EAP (Prilenia), and 10-site Autologous Treg infusion EAP (Rapa), funded under the Accelerating Access to Critical Therapies for ALS Act (ACT for ALS). As the MGH site principal investigator, she leads Phase 1, 2, and 3 clinical trials evaluating novel therapeutics for genetic ALS. Dr. Babu serves on the MGH Healey Center Coordinating Center design and operations committee and serves as regimen co-lead for Regimens B and G (Biohaven and Denali) of the Healey ALS platform trial. She serves as faculty of the MGH clinical coordinating center for the gene therapy consortium for gene-based clinical trials conducted within the NINDS NeuroNEXT network. As of March 2024, she serves as a compensated consultant for uniQure, a gene therapy company. . Dr. Babu has also previously consulted with Medscape and served on a Biogen advisory board. Dr. Babu co-chairs the Northeast Amyotrophic Lateral Sclerosis (NEALS) Consortium imaging subcommittee, an international organization for ALS researchers. Her research is funded by awards from the National Institute of Neurological Disorders and Stroke of the National Institutes of Health. Dr. Babu is one of the multiple principal investigators leading the Access for All in ALS (ALL ALS) East Clinical Coordinating Center awarded by NINDS to MGH funded under the ACT for ALS. Dr. Babu completed her neurology training at the Cleveland Clinic, Ohio, and two fellowships in neuromuscular medicine and neurodegenerative disorders at Harvard Medical School. Her clinical research training includes the completion of the following: a 2-year competitive Clinical and Translational Research Academy program at Harvard Medical School (2018), master's in public health at University of Maryland School of Public Health (2008), and a 1-year competitive NIH-funded Clinical Trial Methodology Course (2017 cohort).

Chelsey R. Carter

Member

Chelsey R. Carter is an Assistant Professor of Public Health in the Department of Social and Behavioral Sciences at Yale School of Public Health. Dr. Carter has expertise in medicine, public health, and race. Her research program examines how scientific knowledge production, clinical care, and systemic marginalization impact historically underrepresented communities affected by neurodegenerative diseases, like ALS, and genomic medicine. Dr. Carter is also undertaking a book project which includes an ethnographic study of the diverse experiences of living with ALS, which draws on over 15 years of experience with Black communities affected by ALS. Her scholarship has been funded by the National Science Foundation, the Andrew Mellon Foundation, the Wenner Gren Foundation, the Ford Foundation, the ALS Association, the National Institutes of Health and more. She serves as an executive board member for the Society for Medical Anthropology and from April 2022 – January 2023, Dr. Carter was a working group member for the NIH/NINDS Strategic Plan on ALS. Dr. Carter has provided consulting services or made presentations to pharmaceutical companies and non-profit organizations on issues of race, equity, and inclusion since 2020. Dr. Carter serves on the Care Services Committee for the ALS Association. Dr. Carter was a Presidential Postdoctoral Research Fellow at Princeton University in the Department of Anthropology and Center for Transnational Policing. She received her Bachelor's degree in Anthropology with a minor in Spanish (high honors) from Emory University. She holds a Ph.D. in Sociocultural Anthropology, a Master of Public Health, and Certificate in Women, Gender, and Sexuality Studies from Washington University in St. Louis.

Maceo Carter

Member

Maceo Carter is 47 years old and was diagnosed with ALS six years ago, on November 8, 2016. Two things he has learned during this ALS journey are: (1) each person's journey is their own roller coaster filled with highs and lows, and (2) with the right amount of help it is amazing the life you can live, albeit with a terminal disease. Maceo believes all things happen for a reason and that he has been given the opportunity to help with something that seems insurmountable. He wants to change the outlook on ALS, the approach to caring for those living with ALS, and help people live the best life possible with this disease. Maceo works with the ALS Association and the Many Shades of ALS Team at I AM ALS. He is the father of four sons ages 24, 15, 13 and two. He is still employed and works 40 hours a week. Before diagnosis, Maceo thoroughly enjoyed cooking especially with his sons. He is a huge University of North Carolina basketball fan. He and his wife have known each other for over 20 years and have been married for nine.

George Demiris

Member

George Demiris is a Penn Integrates Knowledge (PIK) University Professor in the School of Nursing with a joint appointment in the Department of Biostatistics, Epidemiology and Informatics in the Perelman School of Medicine, University of Pennsylvania. His research explores innovative ways to utilize inclusive technology and support patients and their families in various settings including homes and communities. He has conducted clinical trials to examine telehealth-based interventions for family caregivers in hospice. He also studies “smart home” solutions and digitally augmented residential settings to facilitate passive monitoring via telehealth, and support quality of life for older adults and people with disabilities. He is a Member of the National Academy of Medicine, a Fellow of the Gerontological Society of America and the American College of Medical Informatics. He served on the National Academies committee on the Health and Medical Dimensions of Social Isolation and Loneliness in Older Adults (2020) and a National Academy of Medicine 2018 work group on technologies to enhance person, family and community activation. He has presented his research at a National Academies Forum on Aging, Disability & Independence Workshop on the Role of Human Factors in Home Healthcare, and served as reviewer for the National Academies report, The Promise of Assistive Technology to Enhance Activity and Work Participation (2017).

John Dunlop

Member

John Dunlop is Chief Scientific Officer, Aliada Therapeutics, a central nervous system-focused drug development company advancing a differentiated approach for blood-brain barrier delivery of a range of biologics therapeutics. Until August 2023, he was head of Research and Development at Neumora Therapeutics, a company launched to pursue an innovative approach to precision medicines for brain diseases. Prior to Neumora, John was at Amgen, where he led the neuroscience research program responsible for therapeutic discovery activities in neurodegenerative diseases, pain and migraine. Prior to Amgen, he led neuroscience discovery and early development at AstraZeneca and previously held executive leadership roles in neuroscience at Wyeth and Pfizer. John is an industry member of the HEAL (Helping End Addiction Long term) Partnership Committee, a National Institutes of Health (NIH) advisory committee established to support NIH initiatives launched to address the nation’s opioid crisis. He is a board member of Target-ALS, a non-profit enterprise dedicated to accelerating drug discovery and development in ALS, and the Massachusetts Biotechnology Council (MassBio), and is on the scientific advisory boards of Vigil Neuroscience and the Packard Center for ALS Research at Johns Hopkins. John holds a B.Sc. in biochemistry from the University of Glasgow and a Ph.D. in neuroscience from the University of St. Andrews.

Eva L. Feldman

Member

Eva L. Feldman is the James W. Albers Distinguished University Professor, the Russell N. DeJong Professor of Neurology and Director of the ALS Center of Excellence at the University of Michigan (ALS Centers of Excellence receive some funding from the ALS Association to support patient care). With 30 years of continuous funding from the National Institutes of Health, she was the principal investigator on the first two FDA-approved stem cell transplant clinical trials for ALS. Related to her work in stem cell transplant treatments for ALS, Feldman previously worked with biotech companies (e.g., NeuroStem Inc. and Seneca) on the clinical translation of her research. Feldman also had a one-time consulting relationship with Biogen in 2022 regarding ALS therapies. Feldman recently received an NIH Director's Transformative Award for her groundbreaking research on the ALS exposome, as well as separate grants from NIEHS and CDC for her work on linking environmental pollutants with ALS, and NINDS on how immunity contributes to ALS risk. Since December 2023, she is a participant in the Neural Exposome Strategic Plan Working Group being convened by the NINDS Office of Neural Exposome & Toxicology (ONETOX) to consider high impact exposomic research opportunities. Feldman is an author of the 2022 Lancet ALS seminar outlining the current state of ALS diagnosis, clinical care, and research. At the University of Michigan, she directs a research program of 25 scientists and has over 500 publications. Feldman served as president of the American Neurological Association (2011-13), the third woman to hold this position in 130 years and has received lifetime achievement awards from multiple societies. She is an elected member of the National Academy of Medicine. Feldman received her MD, PhD from University of Michigan, completed neurology residency at Johns Hopkins and a neuromuscular fellowship at University of Michigan.

Holly Fernandez Lynch

Member

Holly Fernandez Lynch is Assistant Professor of Medical Ethics and Law at the University of Pennsylvania. She pursues conceptual and empirical scholarship regarding clinical research ethics and regulation, access to investigational medicines outside clinical trials, and FDA pharmaceutical policy, especially early approval pathways for diseases with unmet treatment needs. She is a board member of Public Responsibility in Medicine & Research (PRIM&R) and the American Society for Law, Medicine, and Ethics, as well as a fellow of the Hastings Center and a National Academy of Medicine Emerging Leader in Health and Medicine. She served as Ethicist in Residence at the Robert Wood Johnson Foundation from 2020-2023, and, as of January 2024, serves on the Observational Study Monitoring Board (OSMB) for the new ALL ALS consortium. She has previously worked as an attorney in private practice, a bioethicist serving NIH's Division of AIDS, an analyst with President Obama's Commission for the Study of Bioethical Issues, and executive director of Harvard Law School's bioethics and health law research program. She earned graduate degrees in law and bioethics at the University of Pennsylvania.

Ileana Howard

Member

Ileana Howard is a physical medicine & rehabilitation physician specializing in the care of persons with ALS. She currently serves as the Chair of the ALS Executive Committee for the Veterans Health Administration as well as caring for patients and families as the Medical Co-Director of the ALS Center of Excellence at the VA Puget Sound in Seattle, Washington. Dr. Howard is an Associate Professor of Rehabilitation Medicine at the University of Washington. She has volunteered for the board of the American Association of Neuromuscular and Electrodiagnostic Medicine (AANEM) and the ALS Association Evergreen Chapter. She was awarded the Clinical Excellence Award by the Paralyzed Veterans of America (PVA) in 2018, and the Advocacy Award by the AANEM in 2021. She has previously received honorarium for her work with AANEM, PVA, and for consulting on a PBS film on individuals living with ALS. Dr. Howard also serves on the national Medical Advisory Board and local Evergreen Chapter Leadership Council for the ALS Association. Dr. Howard is currently providing her expertise as part of an AANEM effort to develop expert-based consensus guidelines for an ALS home health and durable medical equipment medical standard. She has numerous publications and national presentations on the rehabilitation management of ALS. Dr. Howard received her undergraduate degree at Smith College and received a Fulbright fellowship to study public health in Spain prior to completing her medical doctorate at Harvard Medical School. She completed an Internal Medicine internship at the Lahey Clinic, followed by Physical Medicine & Rehabilitation residency at the University of Washington. She maintains dual board certifications in physical medicine and rehabilitation and electrodiagnostic medicine.

Jerome E. Kurent

Member

Jerome Kurent is Professor of Neurology and Medicine at the Medical University of South Carolina and director of the ALS Interdisciplinary Clinic at the Ralph H. Johnson Veterans Affairs Medical Center in Charleston, South Carolina. His primary research and educational outreach have focused on palliative and end-of-life care for patients with incurable neurological diseases including ALS, as well as public policy related to providing care for persons with ALS. His research has also included a focus on improving quality of life for African-American patients with incurable end-stage diseases. Dr. Kurent is co-editor of Public Policy in ALS/MND Care: an International Perspective and A Clinician's Guide To Palliative Care. He served as a member of the National Hospice and Palliative Care Organization ALS Work Group. Honors and Awards include the Lifetime Achievement Award from the International Neuropalliative Care Society (2021); Faculty Scholar of the Project on Death in America; and Fellow, Mayday Pain and Society. Dr. Kurent received his MD from the University of Cincinnati, MS from Ohio State University and MPH from the Harvard School of Public Health. He completed residencies in Neurology and Internal Medicine at the Johns Hopkins Hospital. He completed fellowships in Neurovirology, Neuromuscular Diseases and Electromyography at the National Institutes of Health and in Geriatric Medicine from Harvard.

Won Young Lee

Member

Won Young Lee is an associate professor in the Division of Pulmonary and Critical Care Medicine at the University of Texas Southwestern Medical Center in Dallas. Dr. Lee serves as Medical Director of the Clinical Center for Sleep and Breathing Disorders Center and has worked at UT Southwestern for 15 years as an academic faculty, in the clinician educator track. Dr. Lee is board certified in internal medicine, pulmonary medicine, critical care medicine, and sleep medicine. Dr. Lee brings pulmonary expertise in managing patients with chronic respiratory failure relevant to neuromuscular conditions, including ALS, muscular dystrophy, post polio syndrome, spinal cord injury, diaphragm disorders, myasthenia gravis, and inflammatory muscle or neurologic conditions. In addition, he has expertise in sleep medicine, and continues to practice both pulmonary and critical care medicine. Dr. Lee has received numerous teaching awards including, most recently, the Regents Outstanding Teaching Award for the State of Texas in 2019. Dr. Lee received his medical degree from University of Nevada Reno School of Medicine and completed internal medicine residency at University of Nevada Affiliated Hospitals. Dr. Lee completed a Pulmonary and Critical Care Fellowship at Rush University Medical Center, Chicago, and a Sleep Medicine Fellowship at the University of Chicago.

Harold L. Paz

Member

Harold Paz is an operating partner at Khosla Ventures focused on healthcare and life science companies. Previously, Dr. Paz was professor of medicine at the Renaissance School of Medicine, Stony Brook University where he was also Executive Vice President for Health Sciences and Chief Executive Officer of Stony Brook University Medicine. Dr. Paz has also served as Executive Vice President and chancellor for health affairs at Ohio State University and Chief Executive Officer of the Ohio State Wexner Medical Center. Before joining OSU, Dr. Paz was Executive Vice President and Chief Medical Officer at CVS Health/Aetna, where he led clinical strategy and policy for Aetna's domestic and global businesses. He has served on numerous healthcare and biotechnology boards and is currently a member of the National Academy of Medicine Leadership Consortium, the board of directors of Research!America and Envision Health, and the medical advisory board of Curai Health. Dr. Paz previously served on the Board of Directors for Select Medical, which provides rehabilitation and post-acute care services, and United Surgical Partners International, an ambulatory surgery company. Dr. Paz received his bachelor's from the University of Rochester, a master's in life science engineering from Tufts University, and his medical degree from the University of Rochester. He completed his residency at Northwestern University. Dr. Paz was a Eudowood Fellow in pulmonary and critical care at Johns Hopkins Medical School and a post-doctoral fellow in environmental health science at Johns Hopkins School of Hygiene and Public Health.

Tonya Roberts

Member

Tonya J. Roberts is an Associate Professor and Karen Frick Pridham Professor in Family-Centered Care in the School of Nursing at the University of Wisconsin – Madison. Dr. Roberts' research is focused on optimizing person- and family-centered care to improve quality of life for vulnerable older adults who require long-term care, with a particular emphasis on promoting autonomy, dignity, and meaningful living in nursing homes. Her research focuses on identifying ways to increase patient and family engagement in care planning and aligning care with patient and family preferences, priorities, and goals. She has prior experience in direct care, nursing, and nursing leadership roles in long-term care settings. She is an Affiliate of the Center for Aging Research and Education at the University of Wisconsin and a member of Advancing Excellence, the Moving Forward Coalition, and the Gerontological Society of America. Dr. Roberts received a BS, MS, and PhD from the University of Wisconsin – Madison and completed a Postdoctoral Fellowship with the Department of Veterans Affairs. She has formal training in nursing, industrial and systems engineering, and health services research.

Rita Sattler

Member

Rita Sattler is Professor of Translational Neuroscience at the Barrow Neurological Institute in Phoenix, Arizona. Her research is focused on mechanisms of neurodegeneration in varying dementias, including frontotemporal dementia (FTD), FTD with motor neuron dysfunction (FTD/ALS), Alzheimer's disease, and Lewy Body dementia using human model systems, including postmortem autopsy tissues and human patient-derived induced pluripotent stem cells differentiated into neurons and glial cells. Dr. Sattler is a member of the American Society for Neuroscience and the American Society for Neurochemistry. She is the recipient of several awards and fellowships, including the Governor's Gold Medal for the highest academic achievement in graduate studies at the University of Toronto and a Human Frontier Science Program Long-term Fellowship. Dr. Sattler serves in uncompensated, advisory roles for the Robert Packard Center for ALS Research, Milken Foundation, LiveLikeLou Foundation, Spinogenix Inc., and Korro Bio Inc. Dr. Sattler serves on the Scientific Advisory Boards of the Northeast Amyotrophic Lateral Sclerosis (NEALS) Consortium and a new scientific program on FTD sponsored by the Milken Institute and the Kissick Family Foundation. In addition, Dr. Sattler has worked with Regenesys Biomedical Inc. Dr. Sattler received her masters and doctorate degree from the University of Toronto and performed her postdoctoral training in the Department of Neuroscience at Johns Hopkins University. She then served as lead scientist for a small startup company overseeing assay development and drug screening of lead compounds for ALS, in addition to the validation of disease biomarkers. From there, Dr. Sattler joined the Drug Discovery Center at Johns Hopkins University to strengthen her expertise in preclinical drug development. She started her first faculty position as Assistant Professor of Neurology in 2012 at Johns Hopkins University.

Joel Shamaskin

Member

Joel Shamaskin is Professor Emeritus of Medicine (retired), at The University of Rochester School of Medicine and Dentistry. He served as Chief Resident in Primary Care Internal Medicine, Fellow in Geriatric Medicine, Attending Physician at Strong Memorial Hospital and Site Medical Director in the University of Rochester Primary Care Network. He was a clinical educator and practiced primary care internal medicine for 31 years until his ALS diagnosis in 2016. Since 2017 he has been actively involved in various ALS advocacy efforts. Dr. Shamaskin has previously received honorarium from the ALS Association and the Institute for Clinical and Economic Review related to his work in ALS. He was elected a Fellow of the American College of Physicians in 1996 and was the recipient of the James M. Stewart Outstanding Teaching award from the University of Rochester in 2014. He received his M.D. degree at The University of Virginia in 1980 and completed residency training at The University of Rochester School of Medicine and Dentistry.

Joshua M. Sharfstein

Member

Joshua Sharfstein is vice dean for public health practice and community engagement and professor of the practice in health policy and management at the Johns Hopkins Bloomberg School of Public Health. He formerly served as principal deputy commissioner of the U.S. Food and Drug Administration and secretary of Maryland's Department of Health and Mental Hygiene. He is an elected member of the National Academy of Medicine and fellow of the National Academy of Public Administration. A board certified pediatrician, he graduated from Harvard Medical School in 1996, the Boston Combined Residency Program in Pediatrics in 1999, and the fellowship in General Academic Pediatrics at the Boston University School of Medicine in 2001. Dr. Sharfstein is working on a part-time federal detail to NIH to support efforts to develop a national hepatitis C elimination effort.

Anantha Shekhar

Member

Anantha Shekhar is senior vice chancellor for the health sciences and John and Gertrude Petersen Dean of the School of Medicine at the University of Pittsburgh. He is a nationally recognized educator, researcher, and entrepreneur with major contributions in medicine and life sciences. At Pitt, Dr. Shekhar leads all six health sciences schools that, for more than a century, have led education and research in their respective fields, propelling scientific discovery and clinical innovation that advance human health. Education at the Schools of the Health Sciences—dental medicine, health and rehabilitation sciences, medicine, nursing, pharmacy, and public health—emphasizes individualized curricula and inter-professional team-based instruction to train students in core competencies of their chosen health professions while also preparing them as advocates, innovators, and stewards of responsible, inclusive health care delivery. Innovation, transformation, and successful collaborations across the private, public, and philanthropic sectors have defined Dr. Shekhar's career. His areas of expertise include basic and clinical neuroscience research on the effects of stress, stress-induced neuropsychiatric disorders, and clinical neuropharmacology. His laboratory has developed several translational models for neuropsychiatric disorders as well as identified novel targets for neuropsychiatric disorder treatments such as schizophrenia, major depression, Chronic pain and neurodegenerative disorders that are in various stages of commercial development. Grants from the NIH, private foundations and commercial collaborations have supported his research. He has co-authored more than 250 original scientific papers published in leading basic science and clinical journals. He is a founder of multiple biotech companies developing novel therapies. Dr. Shekhar is a member of the National Academies of Sciences, Engineering, and Medicine's Forum on Drug Discovery, Development, and Translation. Dr. Shekhar, who was born in India, earned his medical degree at St. John's Medical College, Bangalore, and PhD in neuroscience at Indiana University.

Mindy Uhrlaub

Member

Mindy Uhrlaub is an author and Familial ALS activist. She is a pre-symptomatic carrier of the fatal gene, C9orf72, which causes ALS and Frontotemporal Dementia (FTD). She has won several awards for her writing, including the 2021 NYC Big Book Award for Cultural Heritage. For the writing of her forthcoming book, *A War of Nerves: An ALS Memoir*, she has been awarded residencies at Millay Arts, the Hambidge Center, and Joyce Maynard's Write by the Lake. Mindy frequently writes and speaks about access to ALS drugs for pre-symptomatic carriers. She is participating in more than a dozen longitudinal studies of ALS/FTD and is currently enrolled in Synapticure's four-year study of Riluzole for premanifest genetic carriers of ALS. Through ALS/FTD: End the Legacy, an organization that advocates for carriers of genetic ALS, Mindy has testified before the FDA in an opening listening session about the needs of premanifest carriers of ALS genes.

Rebecca English

Staff Officer

Rebecca English, is a Senior Program Officer in the Board on Health Science Policy. She has directed, co-directed, and staffed a number of projects at the National Academies including, most recently, Realizing the Promise of Equity in the Organ Transplantation System (2022), Assessment of Strategies for Managing Cancer Risks Associated with Radiation Exposure During Crewed Space Missions (2021), and Necessity, Use, and Care of Laboratory Dogs at the U.S. Department of Veterans Affairs (2020). She staffed the Forum on Drug Discovery, Development, and Translation at the National Academies in various capacities between 2009 – 2016, working on wide-ranging projects related to the United States clinical trials enterprise as well as multidrug-resistant tuberculosis throughout the world. Prior to joining the National Academies, she worked on health policy for Congressman Porter J. Goss (FL-14) and for the National Active and Retired Federal Employees Association. Rebecca holds an M.P.H. from the University of Michigan and a B.A. from the University of Notre Dame in political science.