

ONE GOAL

Identify a treatment for ALS.

ONE RESEARCH TEAM

The top leaders in ALS research have banded together to leverage the strengths of their venerable institutions into one ambitious plan to bring a treatment to patients within four years. The team:

- Dr. Robert Brown, UMass Medical School
- Dr. Merit Cudkowicz, Mass General Hospital
- Dr. Steven Perrin, ALS Therapy Development Institute
- Dr. Nazem Atassi, Mass General Hospital

ONE PATH

By collaborating to fund and develop a treatment, we will demonstrate our shared commitment to fighting ALS while also continuing to provide high-level care for those who are already diagnosed.

ONE NEED

The ALS ONE scientific plan is milestone driven, and will require significant funding to advance. A percentage of funds raised will be directed to assist patients with the many needs they will face until a treatment is available.

ONE CARE TEAM

A percentage of funds will be used to improve care and the care model in ALS, currently delivered by the following teams:

- Ron Hoffman, Compassionate Cares ALS (CCALS)
- Care staff at Mass General Hospital
- Care staff at UMass Medical Center

ONE PLAN

To raise \$x million through:

- Major gifts
- Business sponsorships
- Crowdfunding / social media
- Small business networks (ALS Knights)
- Non-profit alliances
- **ALS ONE** Day (5.7.16)





Dr. Robert H. Brown, Jr. is Professor and Chair of Neurology at the University of Massachusetts Medical Center and Medical School. He is also the Director of the Day Neuromuscular Research Laboratory at the University Of Massachusetts Medical School.

Dr. Brown graduated from Harvard Medical School and completed his doctoral training in neurophysiology at Oxford University. Dr. Brown trained in Neurology at the Massachusetts General Hospital.

In 1984, The Day Neuromuscular Research Laboratory was founded by Dr. Brown to investigate neuromuscular diseases, including Miyoshi myopathy and ALS.

Dr. Brown's laboratory has focused on the identification of gene defects that elucidate the molecular pathogenesis of selected neuromuscular diseases including amyotrophic lateral sclerosis (ALS, also known as Lou Gehrig's disease), muscular dystrophy, adrenoleukodystrophy, hereditary neuropathy and hyperkalemic periodic paralysis. Knowledge of these disease genes has facilitated the creation of mouse and cell-based models of these disorders. In turn, these resources have allowed study of therapeutic strategies using conventional small molecule approaches and new modalities such as inhibitory RNAi.



Dr. Merit Cudkowicz is the Julieanne Dorn Professor of Neurology at Harvard Medical School, Chief of Neurology Service at Massachusetts General Hospital. Dr. Cudkowicz's research and clinical activities are dedicated to the study and treatment of people with amyotrophic lateral sclerosis (ALS).

Dr. Cudkowicz directs the Massachusetts General Hospital ALS Program and the Massachusetts General Hospital Neurological Clinical Research Institute. She is one of the founders and former co-directors of the Northeast ALS Consortium (NEALS), a group of over 100 clinical sites in the United States, Canada, Europe and the Middle East dedicated to performing collaborative academic led clinical trials and research studies in ALS.

In conjunction with the NEALS consortium, she planned and completed over 15 multi-center clinical research studies in ALS. She is Principal Investigator of the Clinical Coordination Center for the National Institute of Neurological Disorders and Stroke's Neurology Network of Excellence in Clinical Trials (NeuroNEXT). The NeuroNEXT network is an efficient phase II network to develop innovative and new treatments for people with neurological disorders.

Dr. Cudkowicz received the American Academy of Neurology 2009 Sheila Essay ALS award. She is a pioneer in promoting and developing more efficient methods of developing new therapies for people with ALS. A dedicated educator, Dr. Cudkowicz mentors many young neurologists in clinical investigation of ALS and related neurodegenerative disorders. Dr. Cudkowicz completed her undergraduate degree in chemical engineering at Massachusetts Institute of Technology and obtained a medical degree in the Health Science and Technology program of Harvard Medical School. She served her internship at Beth Israel Hospital in New York and her neurology residency and fellowship at Massachusetts General. She also obtained a master's degree in Clinical Epidemiology from the Harvard School of Public Health.



Dr. Steven Perrin is currently the Chief Executive Officer and Chief Scientific Officer at the ALS Therapy Development Institute (ALS TDI) in Cambridge, MA.

He earned his Ph.D. at Boston University Medical Center studying the transcriptional regulation of genes during adipocyte and myocyte differentiation.

Steven moved into the pharmaceutical industry in 1997 holding positions at the Hoechst-Ariad Genomics Center, Aventis Pharmaceuticals and more recently as Director of Molecular Profiling at Biogen Idec.

Steven joined ALS TDI in 2007 as part of historical collaboration between the Muscular Dystrophy Association, Augie's Quest and ALS TDI to develop effective therapeutics for ALS patients. Since joining ALS TDI Steven has spearheaded the development of computational biology capabilities and information management systems to more clearly understand the molecular mechanisms associated with disease onset and progression in neurodegenerative diseases. He has expanded ALS TDI's drug screening program to include expertise in the generation and assessment of gene therapy vectors and protein biologics in preclinical models of neurodegeneration.

He has developed a business plan to facilitate pharmaceutical partnerships for rapid clinical development and commercialization of promising targets for ALS patients.



Dr. Nazem Atassi is Associate Director of the Neurological Clinical Research Institute (NCRI) at Massachusetts General Hospital (MGH) in Boston, Massachusetts, Assistant Professor of Neurology at Harvard Medical School, completed Neurology training at Boston University Medical Center and Fellowship in Neuromuscular Disorders and Clinical Trials at MGH.

Dr. Atassi received his Masters of Medical Science in 2010 from Harvard Medical School. He serves on the executive committee of the Northeast ALS Consortium (NEALS), and he is the founder and Co-Chair of the Upper Motor Neuron and the Imaging committees at (NEALS), and is a board member of the ALS Research Group (ALSRG).

Dr. Atassi received several awards including the MIT 100K Life Science Award from Massachusetts Institute of Technology (MIT), the Anne B. Young Translational Neuroscience Fellowship, and NIH K23 Career Development Award. He has hands-on industry experience in running multi-center clinical trials through his work as a medical monitor for Pfizer and Fellow at Biogen.

Dr. Atassi is the Primary Investigator for several research projects focusing on Amyotrophic Lateral Sclerosis (ALS) clinical trials and neuroimaging. He attends two clinics at MGH: ALS multidisciplinary clinic and Neuromuscular clinic.



Ron Hoffman co-founded the Gordon T. Heald ALS fund along with Elizabeth Heald following the death of her husband Gordon on October 30, 1998. In time Ron's work led to the creation of the nonprofit organization Compassionate Care ALS. He has served as founder and director working with over 1,200 individuals, families and communities living with Lou Gehrig's Disease in the US and around the world. He facilitates and guides intimate discussions for those engaged in the complexities of living with and dying with this catastrophic illness. Ron's work helps examine the myriad of choices and possibilities that people confront including the process of death and dying and the questions that arise as together they create space for dialogue around their decisions.

Ron has been mentored by many great teachers in preparation for his unique path in the world, including Roshi Joan Halifax of the Upaya Institute, the late Steven Foster, his wife Meredith Little and Dr. Scott Eberle of the School of Lost Borders, Jeffrey Duvall, co-founder of the Men's Council Project, Tom Daly, Ph.D. of the Living Arts Foundation and Julie Brown Yau, Ph.D. of Embodied Wisdom who was instrumental in the co-creation of the Cultivating Compassion Education Series. Ron leads in-services and trainings for hospice workers and health care professionals. He also lectures regularly to nursing students at UMASS Boston. He is a former board member of Men's Leadership Alliance of Boulder, Colorado and is a past board member of the Hospice and Palliative Care Federation of Massachusetts.

Ron is the author of *Sacred Bullet, Transforming Trauma to Grace While Tending the Terminally Ill*, a book that sheds light on living with grace in the face of mortality. Ron serves in many ways as a resource for individuals and organizations around the country. Originally born in Richmond, Virginia, he currently lives in West Falmouth, Massachusetts, with Moses, his Bernese mountain dog.