

**Harvard Medical School
Curriculum Vitae**

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Place of Birth: Oak Ridge, Tennessee

Education

1985	B.S. Tau Beta Pi	Chemical Engineering	Massachusetts Institute of Technology
1990	M.D.	Medicine	Harvard Medical School, Harvard- Massachusetts Institute of Technology. Division of Health, Science and Technology
1996	MSc.	Clinical Epidemiology	Harvard School of Public Health

Post Graduate Courses

2010-2012	Physician Leadership Development Certificate Program	Massachusetts General Hospital
2013	Program for Chiefs of Clinical Service	Harvard School of Public Health

Postdoctoral Training

07/90-6/91	Intern	Medicine	Beth Israel Hospital
07/91-6/93	Resident	Neurology	Massachusetts General Hospital
07/93-6/94	Chief Resident	Neurology	Massachusetts General Hospital
07/94-6/96	Clinical Research Fellow	Neurology	Clinical Investigator Training Program, Harvard/M.I.T. Health Sciences and

Technology-Beth Israel Hospital in collaboration with Pfizer

Faculty Academic Appointments

07/95-6/97	Instructor	Neurology	Harvard Medical School
07/97-6/02	Assistant Professor	Neurology	Harvard Medical School
07/02-12/09	Associate Professor	Neurology	Harvard Medical School
01/10-	Julianne Dorn Professor	Neurology	Harvard Medical School

Appointments at Hospitals/Affiliated Institutions

Past

06/96-4/03	Assistant in Neurology	Neurology	Massachusetts General Hospital
1996-1997	Research Affiliate	General Clinical Research Center	Massachusetts Institute of Technology
2004-2006	Staff Researcher	Neurology	Edith Norse Bedford Veteran's Administration Hospital
1998-	Associate in Neurology	Neurology	Brigham and Women's Hospital
04/03-4/11	Associate in Neurology	Neurology	Massachusetts General Hospital

Current

04/11-	Neurologist	Neurology	Massachusetts General Hospital
05/12-	Chief, Neurology Service	Neurology	Massachusetts General Hospital
6/20 -	Clinical Consultant	Neurology	Brigham and Women's Hospital

Other Professional Positions

2000-2001	Medical Advisory Board	Medtronic
2004	ALS Advisory Board	Jazz Pharmaceutical
2005-2007	Scientific Advisory Board	Teva Pharmaceuticals
2008-2009	Medical Advisory Board	Acceleron
2007-2008	Scientific Advisory Board	Clinical Research Network for Friedrich's Ataxia
2008	Scientific Advisory Board	Ruxton
2009	Scientific Advisory Board	Roche
2009-2011	Scientific Advisory Board	GlaxoSmithKline
2008	Scientific Advisory Board	Ruxton
2012	Scientific Advisory Board	Coyote Pharmaceuticals
2012-2015	Scientific Advisory Board	DART
2016	ALS Endpoint Advisory Board	Biogen
2018	Executive Advisory Committee	P01 SAB

		Pathophysiology of genetically defined Dementia and Neurodegeneration Defining Therapeutic Targets and Pathways
2019-	Science Advisory Board	ALS Therapy Development Fund
2020-	Science Advisory Board	NeuroSense
2020-	Science Advisory Board	BioTrust
2022-	Science Advisory Board	Akava

Major Administrative Leadership Positions

Local

1997-2019	Co-Director, Neurology Clinical Research Institute (NCRI)	Massachusetts General Hospital
1998-2008	Co-Director, Amyotrophic Lateral Sclerosis (ALS) Center	Massachusetts General Hospital
2001-2006	Co-Director, Harvard Center for Neurodegeneration and Repair, Core A Neurologic Clinical Trial Service	Harvard Medical School
2007-2008	Co-Director, Muscular Dystrophy Association Neuromuscular clinic	Massachusetts General Hospital
2008-2016	Director, Muscular Dystrophy Association Neuromuscular clinic	Massachusetts General Hospital
2009-2016	Division Co-Chief, Neuromuscular	Massachusetts General Hospital
2012-2013	Chair, Research Strategy Team Clinical Research	Massachusetts General Hospital
2018-2021	Vice Chair, ECOR	Massachusetts General Hospital
2018	Director, Healey Center for ALS	Massachusetts General Hospital
2019	Chair, Search Committee for Psychiatry	Massachusetts General Hospital
2021-2024	Chair, ECOR	Massachusetts General Hospital
2021	Chair, Search Committee for Director Division Clinical Research	Massachusetts General Hospital
2022-	Chair, Search Committee for Department of Medicine	Massachusetts General Hospital
2023	Co-Chair, Philanthropy	Massachusetts General Hospital

National

2007-2012	Co-Director, Clinical Trial Methods Course	National Institute of Neurological Disorders and Stroke
2007	Co-Chair and Organizer, Banbury ALS Drug Discovery Workshop	ALS Association
2007	Course Director, Selecting Experimental treatments for clinical testing	Huntington's Disease Study Group
2009	Chair and Organizer, ALS Phase 2 Design Workshop	Muscular Dystrophy Association
2014	Chief Medical Officer	ALS Finding A Cure Foundation

International

2008	Co-Director, Biomarkers in ALS Symposium, Birmingham, UK	World Federation of Neurology
2010	Chair, International Motor Neuron Disease Symposium	Motor Neuron Disease Association
2011	Session Chair, ICSCN Workshop	Motor Neuron Disease Association

Committee Service***Local***

1993	Neurology resident selection committee	Massachusetts General Hospital Member
1996-2004	Scientific Advisory Board	General Clinical Research Center, Massachusetts General Hospital Member
1996-1997	Clinical Research Education Unit, Clinical Research Program	Massachusetts General Hospital Member
1999-2007	Clinical Research Council	Massachusetts General Hospital Member
2001-2002	Center of Excellence in Women's Health Committee	Harvard Medical School Member
2001-2004	Admissions Committee	Health Science and Technology Medical School Member
2004-2008	Neurology Department Finance Committee	Massachusetts General Hospital Member
2005-2007	Neurology Department Leadership Committee	Massachusetts General Hospital Member
2006-2007	Women's Health Research Committee	Massachusetts General Hospital Member
2006-2007	Search Committee Sleep Unit Co-Director	Massachusetts General Hospital Member
2007-2010	Clinical Research Steering Committee	Massachusetts General Hospital Member
2007-2010	Neurology Clinical Operations Committee	Massachusetts General Hospital Member
2007-2012	Neurology Research Operations Committee	Massachusetts General Hospital Member
2008-2012	Neurology Clinic Directors Committee	Massachusetts General Hospital Member
2009-2011	Research Compliance Committee	Massachusetts General Hospital Member
2009-	Catalyst Participant and Clinical Interactions Resource Scientific and Resource Review Committee	Harvard Medical School Member
2011	Standing Committee on Conflicts of Interest	Harvard Medical School Member

2011	Harvard Catalyst KL2 Admissions Committee	Harvard Catalyst Member
2011	Committee for appointment of Dr. Christopher P. Cannon as Professor of Medicine	Harvard Medical School Member, Brigham and Women's Hospital
2012-	Massachusetts General Physicians Organization Executive Committee	Massachusetts General Hospital Member
2012	Committee for Professor appointment of Dr. Ronald J. Anderson as Professor of Medicine	Harvard Medical School Member
2012-2013	Ad hoc committee search for Chief of the Department of Otolaryngology	Harvard Medical School Massachusetts General Hospital
2012-	Executive Committee on Research	Massachusetts General Hospital Member
2013-2014	Research and Licensing Commercialization Council	Partners Healthcare Member
2016	Ad hoc committee search for Chief of the Department of Neurosurgery	Harvard Medical School Massachusetts General Hospital
2017	2017 World Neuroscience Springboard Committee	Partners Healthcare Member
2020-	Warren Alpert Foundation Prize	Harvard Medical School

National and International

1996-	Executive Committee	Northeast ALS Consortium
	1996-2015	Co-Chair and Founder
	2016-	Member
1997-2004	Executive Board	ALS Care Member
1999-2001	Steering Committee Riluzole in Huntington's Disease	Huntington Study Group Co-Chair
2000-2003	Steering Committee Minocycline in Huntington's Disease	Huntington Study Group Chair
2000-2018	Muscular Dystrophy Association Research Advisory Committee	Member
2001-2003	Safety Monitoring Committee Creatine in Huntington's Disease	Huntington Study Group Chair
2003-2005	Steering Committee Clinical trial of TCH386	Novartis Member
2005-2008	Data Safety Monitoring Committee Somatokine in Myotonic Dystrophy	National Institute of Neurological Disorders and Stroke (NINDS) Member
2005-	Executive Committee	ALS Research Group
	2007-08	Vice Chair
	2009-11	Chair

2005-2007	Steering Committee Arimoclomol in ALS	Cytrx Inc. Chair
2005-2013	Steering Committee Ceftriaxone in ALS	NINDS Chair
2005-2008	Steering Committee Minocycline in Huntington's Disease II	Huntington Study Group Chair
2005-2006	Data Safety Monitoring Board	NIH Rare Diseases Clinical Research Network, Member
2006	Steering Committee DIMOND Huntington Disease Trial	Mediavation Member
2006	Steering Committee PREQUEL: CoQ for presymptomatic Huntington's Disease	NINDS Member
2007-2008	Data Safety Monitoring Board	Ono Pharmaceuticals Member
2007-2010	Data Safety Monitoring Board	Synapse Biomedical Chair
2007-2008	Advisory Panel for Clinical Research	NINDS Co-Chair
2008-2011	Steering Committee Inosine in Parkinson's Disease	Michael J Fox Foundation for Parkinson's Research (Foundation)
2008-2009	ALS Translational Research Advisory Committee	Muscular Dystrophy Association Member
2008-2010	Steering Committee KNS760704 in ALS	Knopp Neurosciences Inc Chair
2009-2011	Data Safety Monitoring Board	Trophos Chair
2009-2015	Sheila Essey Award: An Award for ALS Research Workgroup	Workgroup Member
2010	Therapy Development Committee for Spinal Muscular Atrophy	NINDS Member
2011	Organizing Committee Tarrytown II ALS Conference to promote clinical research to find the cure for ALS.	NINDS
2011	FDA Mock Advisory Panel	Millenium
2014-2018	Data Safety Monitoring Board	Lilly Pharmaceuticals Chair
2015-	Executive Committee Tirasemtiv Phase III Trials in ALS	Cytokinetics Member
2015-2019	Steering Committee Retigabine in ALS	ALSA/ALSFAC/GSK Member
2015-	Steering Committee ALS SOD1 Phase 1 233AS101	Biogen Member
2015-	Publication Committee	Cytokinetics Member
2016-2023	Peripheral and Central Nervous System Drugs Advisory Committee	FDA Member

2016-	Advisory Council for the Brown Institute for Brain Science (BIBS)	Brown University
	2016-2019	Member
	2019-2022	Permanent Member
2016-	ALS Scientific Communication & Education External Expert Steering Committee	Mitsubishi Tanabe Pharma/MTPA Chair
2016-2020	Steering Committee	Amylyx
	AMX0035 for ALS	Member
2016-2020	Steering Committee	NINDS
	Sure PD-3 Clinical Trial	Member
2017	ALS Advisory Board	Biogen
	C9orf72 ASO Phase 1 Study	Member
2017	Scientific Education and Communication Steering Committee	MT Pharma Chair
2018-	Neurobiology Sub-Committee Quarterly Sub-Committee Meeting	Warren Alpert Foundation Chair
2020	Steering Committee Member	AB Sciences
2019-	Intramural Review Committee, Member	NINDS
2021	The EU Academy of Sciences (EUAS)	Member
2020-2024	Committee for The Board of Scientific Counselors for NINDS 7/6/2020-6/30/2024	Member
2021-	Association of American Physicians	Councilor
2022-	American Brain Foundation	Board Member
2023-	American Academy of Neurology	FAAN Member
2023	Alzheimer's Clinical Trials Consortium	Advisory Board Member

Professional Societies

1983-	Sigma Xi Research Society	Member
1983-	Tau Beta Pi National Engineering Honor Society	Member
1990-	American Academy of Neurology	
	2001-2008	Abstract Reviewer
	2005-2008	Chair, Anterior Horn Topic
	2005-2009	Member, Clinical Research
	2009-2011	Subcommittee
	2009-2011	Chair, Clinical Research Subcommittee
	2009-2011	Member, Science Committee
		Member, Translational Neuroscience
	2009-2013	Subcommittee
	2009-2013	Chair, AAN Foundation Research Council
	2009	Member, Sheila Essey Award Subcommittee
	2011-2012	Member, Presidential task force on NIH
	2013	Multi-institutional Collaborations Chair, Fellowship Workforce
		AAN Foundation Research Council
	2015-2020	ABF Research Advisory Committee

Member

1994- 1998-2003 2001- 2006-2010	Huntington Study Group Society for Neuroscience World Federation of Neurology	Member Member
2001-2015 2001-2002	The Amyotrophic Lateral Sclerosis Association (ALSA)	Member, Motor Neuron Disease Executive Committee Member, Massachusetts Chapter Scientific Advisory Board Neuroepidemiology consultant
2005- 2005-2007 2006-2009 2007-2009 2013	The American Neurological Association	Member, Scientific Program Committee Member, Clinical Trial Summer Course Committee Member, Long Range Planning Committee Member, Scientific Program Advisory Committee
2009- 2009- 2010-2018	The American Society of Experimental NeuroTherapeutics (ASENT)	Member Editorial Board of <i>NeuroTherapeutics</i>
2011 2016	American Federation for Medical Research American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM)	Member Honorary Member

Grant Review Activities

2000-2018	Medical Advisory Committee Review Panel	Muscular Dystrophy Association Permanent Member
2009-2013	Venture Philanthropy Advisory Grant Review Board	Muscular Dystrophy Association Member
2002	NINDS Special Emphasis Panel	National Institutes of Health Ad hoc Member
2002-2003	NSD-K Clinical Trial Review Panel	National Institutes of Health Ad hoc Member
2003	Peer Reviewed Medical Research Program	Department of Defense

2003-2008	Scientific Review Committee	Reviewer The Amyotrophic Lateral Sclerosis Association Member
2005-2011	AAN Clinical Fellowship Review Committee	American Academy of Neurology
	2005-2009	Member
	2009-2011	Chair
2006-2008	Scientific Review Committee	Les Turner ALS Foundation Member
2007	Scientific Review Committee	Spastic Paraplegia Foundation Member
2009-2010	Scientific Review Committee	AriSLA Foundation of Italy Reviewer
2009-2010	ALS Research Program Integration	Department of Defense Reviewer
2010-2011	Myopathies Scientific Council	Association Francaise Reviewer
2010	Brain Bank Review Committee	Veteran's Affairs Reviewer
2011	Scientific Review Committee Advisory Board	Thierry Latran Reviewer
2013-2015	Independent Review Committee TARGET ALS	Columbia University/P2ALS Review Board Member
2014	Scientific Review Committee	Weston Institute Reviewer
2016-2018	Scientific Review Committee	Joint FTD and AD Drug Foundation Reviewer/Member

Editorial Activities

Ad hoc Reviewer

Annals of Neurology
Brain
Journal of Neurology and Neuropsychiatry
Lancet Neurology
Muscle and Nerve
Neurology
Neuroscience
New England Journal of Medicine

Other Editorial Roles

2008-	Editorial Board Member	ALS Journal
2009-2010	Editorial Board Member	Neurological Bulletins
2010-2018	Editorial Board Member	ASENT <i>NeuroTherapeutics</i>
2011	Editorial Board Member	Journal of Huntington's Disease
2012-	Editorial Board Member	JAMA Neurology
2012	Editorial Board Member	Cureus.com
2013	Editorial Board Member	Annals of Clinical and Translational
2021	Associate Editor	Annals of Neurology

Honors and Prizes

2003	Angel Fund Award in ALS Research	
2006	Wings Over Wall Street MDA ALS Research Award	
2009	Sheila Essey AAN ALS Research Award	American Academy of Neurology
2012	Prize4Life Pioneer Award Leonard Gerson Distinguished Scholar Award	
2013	Distinguished Scholar Award	University of Pittsburgh
2015	Joseph B. Martin Dean's Leadership Award Advancement of Women Faculty	Harvard Medical School
2015	Partners in Excellence Award	Massachusetts General Hospital
2017	Honorary Membership in American Association of Neuromuscular & Electrodagnostic Medicine	AANEM
2017	Forbes Norris Award	International Alliance of MND
2018	Pinnacle Award	Boston Chamber of Commerce
2019	Nominated for Membership	Association of American Physicians
2019	Raymond D. Adams Lectureship Award	American Neurological Association
2020	Diamond Award Honoree	Muscular Dystrophy Association Wings Over Wall Street Committee
2020	One of the top 10 people Transforming Healthcare	Business Insider
2020	Election to membership in the National Academy of Medicine	National Academy of Medicine
2021	Top 10 Clinical Research Achievement Award Centaur Trial for ALS Study	Institute for Clinical Research and Translational Science Institute Tufts Medical Center
2021	Election to Membership to the EU Academy of Sciences	Member
2021	Pillars of Excellence Award	Mass General Brigham
2021	Merit of Distinction Award For abstract "Long-Term Survival of Participants in the CENTAUR Trial of AMX0035 for ALS"	American Academy of Neurology and The Science Committee
2022	Rebecca Luker Courage Award	Target ALS team
2022	Morris ALS/MND Principles Accolade Certificate	The Morris ALS Principles Team
2023	MDA Legacy Award for Achievement in Clinical Research	Muscular Dystrophy Association (MDA)

Report of Funded and Unfunded Projects

Funding Information

Past

- 1994-1996 **Therapeutic Trial of Free Radical Scavengers in Amyotrophic lateral sclerosis**
Pharmaceutical Manufacturer's Association Clinical Pharmacology Fellowship
PI (Fellow)
The major goal of this investigator led study was to determine safety, pharmacokinetics and efficacy of procysteine in people with amyotrophic lateral sclerosis.
- 1994-1996 **Clinical Investigators Training Program Fellowship, Harvard/M.I.T. Health Sciences and Technology**
Beth Israel Hospital, in collaboration with Pfizer
PI (Fellow)
- 1996-1998 **Genetic Epidemiology and Therapeutic Strategies in ALS**
American Academy of Neurology Education and Research Foundation
Clinical Research Training Fellowship (First awardee)
PI (\$70,000)
The major goal of the study was to determine risk and prognostic factors for ALS
- 1996-2001 **Risk Factors and Therapeutic Strategies in ALS**
NINDS K08 NS001896
PI (\$443,458)
This was a mentored training grant to study risk factors for ALS and to test novel treatments in people with ALS.
- 1997-1999 **Pathogenesis of ALS in SOD-1 Transgenic Mice**
The Amyotrophic Lateral Sclerosis Association (Foundation)
PI (\$18,004)
The major goal of the study was to determine whether approaches to decrease oxidative toxicity were effective in an animal model of ALS.
- 1997-1999 **Pathogenesis of ALS in SOD-1 Transgenic Mice**
Muscular Dystrophy Association (Foundation)
PI (\$144,397)
The major goal of the study was to determine whether approaches to decrease oxidative toxicity were effective in an animal model of ALS (supplementary).
- 1997-2001 **Remacemide and Coenzyme Q₁₀ Therapy in Huntington's Disease**
National Institute of Health NINDS R01 NS35284
Site PI (\$90,575)
This was an investigator led 2X2 factorial design clinical trial of remacemide and coenzyme Q₁₀ in over 300 patients with Huntington's disease.
- 1998-2000 **Determinants of disease severity in ALS**
Massachusetts General Hospital Claflin Award
PI
The study goal was to determine clinical and biological factors that affect prognosis in ALS.
- 1998-2000 **Clinical trial of intrathecal BDNF in ALS**
Amgen (Industry)
Site PI (\$405,229)
The study goal was to determine the long term safety and efficacy of BDNF in ALS.
- 1998-2001 **Pathogenesis of ALS in SOD-1 Transgenic Mice (Brown, R)**
NINDS R01 NS36640
Co-PI (\$245,662)
The major goal of the study was to determine whether approaches to decrease oxidative

- toxicity were effective in an animal model of ALS.
- 1998-2001 **Studies of ALS genes and analysis of genes and candidate therapies in ALS (Brown, R)**
The Amyotrophic Lateral Sclerosis Association (Foundation)
Co-PI (\$100,000)
To find new ALS genes.
- 1999-2001 **Clinical trial of subcutaneous brain derived neurotrophic factor (BDNF) in ALS**
Regeneron (Industry)
Site PI (\$95,919)
This was a phase 3 clinical trial of BDNF in people with ALS.
- 1999-2001 **Riluzole Dosing in Huntington's Disease (Marshall, F)**
Orphan Products Development Office, FDA
Co-PI
This was an academic led phase 2 safety and dose finding study of Riluzole in people with Huntington's disease.
- 1999-2002 **Clinical trial of Topiramate in ALS**
Ortho-McNeil Pharmaceutical (Industry)
Study PI– Investigator initiated Clinical Trial (\$248,704)
MGH Neurology Clinical Trials Unit – Coordination and Data Management Center
This was an academic led multi-center phase 2/3 clinical trial of Topiramate in ALS.
- 1999-2002 **Clinical trial of Topiramate in ALS**
Muscular Dystrophy Association (Foundation)
PI (\$98,781)
To test Efficiency and safety of Topiramate in ALS.
- 1999-2003 **Creatine therapy in ALS (Shefner, J)**
Avicena & Muscular Dystrophy Association (Foundation)
Co-PI (\$34,606)
This was an academic led, multi-center phase 2 clinical trial of Creatine in ALS.
Avicena supplied study drug and supplementary funding.
- 1999-2003 **Clinical Trial of Creatine in ALS**
MDA/Johns Hopkins University Subcontract
PI (\$54,799)
This was an academic led, multi-center phase 2 clinical trial of Creatine in ALS. Funding was obtained from MDA and Avicena.
- 2000-2001 **Open-label remacemide clinical study in Huntington's Disease**
Astra Pharmaceuticals (Industry)
Site PI (\$95,400)
Clinical trial of remacemide in Huntington's Disease
- 2000-2004 **Clinical trial of Topiramate in ALS**
National Institute of Health NINDS R01 NS39988
Pilot Clinical Trial Grant
PI (\$1,073,193)
This was an investigator initiated clinical trial (20 sites) of Topiramate in people with ALS.
- 2001 **Treatment of transgenic ALS mice with a novel cytokine (Brown, R)**
Biogen (Industry)
Co-PI (\$72,262)
Develop treatments for ALS.
- 2001-2002 **Dextromethorphan/quinidine treatment for pseudobulbar affect in ALS**

- Avanir Pharmaceuticals (Industry)
Site PI (\$29,470)
The study goal was to determine the symptomatic effects of dextromethorphan/quinidine on pseudobulbar affect in patients with ALS.
- 2001-2002 **Treatment of ALS mice with ONO-2506** (Brown, R).
Ono Pharmaceuticals (Industry)
Co-PI (\$49,712)
The study goal was to determine efficacy of ONO-2506 in a mouse model of ALS.
- 2001-2003 **Clinical trial of Minocycline in HD**
Orphan Product Development Grant, FDA FDR-001968
PI (\$592,188)
This was a phase 2 investigator initiated clinical trial (8 sites) of Minocycline in 60 people with Huntington's disease.
- 2001-2004 **Clinical Trial of Celebrex in Subjects with ALS** (Drachman, D).
Muscular Dystrophy Association (Foundation)
Co-PI (\$158,680)
The study was an investigator initiated phase 2/3 clinical trial (25 sites) of Celebrex in 300 people with ALS (supplementary).
- 2001-2005 **HCNR Core A Clinical Research**
Harvard Medical School
PI (\$706,450)
Funds were provided to build clinical research infrastructure at Partners.
- 2002-2004 **P35 caspase inhibition in an ALS mouse model**
Muscular Dystrophy Association (Foundation)
PI (\$116,073)
Develop treatment for ALS.
- 2003-2004 **Pilot Safety and Tolerability Study of Coenzyme Q10 in Huntington's Disease and Normal Subjects**
University of Rochester Subcontract
Co-PI (\$35,300)
This was an academic led phase 2 study to determine the maximum tolerated dosage of Coenzyme Q10 in preparation for a subsequent phase 3 efficacy study.
- 2003-2005 **Clinical trial of TCH386 in ALS**
Novartis (Industry)
Site PI and Steering Committee Member (\$119,906)
The study was a phase 2 clinical trial of four dosage of TCH386 in 500 people with ALS.
- 2004-2006 **Metabolomics in ALS**
NIEHS/R21 ES013470
PI (\$280,506)
The main goal was to study the metabolomic profile in blood and cerebrospinal fluid from people with ALS to better understand disease pathways and targets.
- 2004-2006 **Identification of Diagnostic Biomarkers and Therapeutic Targets for ALS**
ALS Association
PI (\$45,000)
The main goal was to study the metabolomic profile in blood and cerebrospinal fluid from people with ALS to better understand disease pathways and targets.
- 2004-2007 **Metabolomics and proteomics in ALS**
The Amyotrophic Lateral Sclerosis Association (Foundation)
PI (160,000)

- The study goal was to build a biobank of well characterized samples and clinical data from people with ALS and controls for biomarker studies.
- 2004-2007 **Safety and tolerability study of sodium phenylbutrate in ALS**
VA Grant, Bedford VA
CoPI (PI Ferrante)
- 2004-2014 **Clinical Trial of Ceftriaxone in Patients with ALS**
NINDS U01 NS049640
Overall Study PI (\$32,737,110)
This is an academic led, adaptive design phase 1-3 investigator initiated clinical trial (62 sites) to determine the safety, dosage and efficacy of Ceftriaxone in people with ALS. MGH is the Coordination and Data management center for this trial.
- 2005-2006 **Safety and tolerability study of sodium phenylbutrate in ALS**
Muscular Dystrophy Association (Foundation)
PI (\$140,417)
The MDA provided supplementary funds to cover laboratory costs for this phase 2 academic led clinical trial.
- 2005-2006 **ALS Research Group DNA Banking Study**
NINDS, Supplement NS 049640-02S1
Co-PI (\$38,661)
The main goal was to create a bank of DNA samples from patients with ALS for research.
- 2005-2006 **Double-Blind, Randomized, Placebo-Controlled, Phase 1 Multiple dose Study to evaluate the safety, tolerability and Pharmacokinetics of AEOL 10150 Administered by Subcutaneous Injections in Patients with ALS**
Aeolus Pharmaceuticals, Inc
Site PI and Steering Committee Member (\$157,766)
This study was designed to test the safety and pharmacokinetics of multiple doses of an antioxidant, AEOL 10150. MGH was a site in this trial.
- 2005-2007 **Phase IIa clinical study of Arimoclomol in ALS**
CytRx Corporation (Industry)
PI (Overall PI, Coordination and Data Management Center) \$1,015,797
This trial was a multi-center, safety, pharmacokinetic, dose finding study of Arimoclomol, an activated of heat shock factor, in people with ALS.
- 2005-2007 **Clinical Trial of High Dose Coenzyme Q10 in ALS**
NINDS/Columbia University Subcontract
Co-Site Investigator (\$5,902)
The goal of this academic led phase II was to determine whether Coenzyme Q10 was worth taking forward to a phase III trial (futility design study). MGH was a site in this study.
- 2005-2016 **Coenzyme Q10 in Huntington's Disease**
NINDS U01 NS52592
Overall Study PI (\$7,660,189)
The study is an investigator initiated phase 3 clinical trial (46 sites) of 2400 mg/day of Coenzyme Q10 in 608 people with Huntington's disease.
- 2006-2007 **Quantitative strength measurement development for ALS**
The Amyotrophic Lateral Association (Foundation)
PI (\$83,193)
The main study goal was to build a new apparatus to quantitatively measure strength in People with neuromuscular disorders.

- 2006-2008 **ALS Research Group DNA Banking Study**
Muscular Dystrophy Association (Foundation)
Co-PI (\$91,826)
The study goal was to gather DNA and clinical data from large numbers of patients with ALS for a national sample bank.
- 2006-2008 **Validation of ALS Metabolomic Biomarkers and Development of ALS Diagnostic**
The Amyotrophic Lateral Association (Foundation)
PI
The main goal of this study was to develop a biomarker for ALS building on results from Our prior metabolomic studies. This was a collaborative project with Metabolon, Inc.
- 2006-2011 **Specialized Program of Translational Research in Acute Stroke (SPOTRIAS) Data Management Core**
NINDS, P50 NS051343
Co-PI, Biostatistics and Data Management Core (\$485,797)
The main goal is to provide data management support for all funded SPOTRIAS stroke project at MGH.
- 2006-2011 **Antisense oligonucleotide therapy for SOD1 mediated ALS**
The Amyotrophic Lateral Association (Foundation)
PI (\$300,000)
This is an investigator-initiated phase 1 gene therapy trial for patients with familial ALS caused by mutations in superoxide dismutase 1. Leadership is shared with Dr. Tim Miller at Washington University.
- 2007-2009 **Development of Huashan and Beijing Hospitals as an ALS Clinical Trial Sites**
Inter-Asia Management, Ltd. (Industry)
PI (\$38,991)
The main goal of this project was to educate and train ALS centers of excellence in care and clinical trial conduct in China.
- 2007-2010 **Validation of a new quantitative strength measurement device for ALS**
Muscular Dystrophy Association (Foundation)
PI (\$201,398)
The main study goal is to develop the prototype for new and better method to quantitate strength in ALS clinical trials.
- 2007-2010 **Predict and Prevent ALS Study**
Muscular Dystrophy Association (Foundation, Emory Subcontract)
Co-PI (\$39,908)
The study goal is to determine, using several outcome measures, the first sign of ALS in people who carry mutations in Superoxide dismutase. This data will be used to design future preventative clinical trials.
- 2007-2011 **Translational Research Grant to develop antisense oligonucleotide therapy for SOD1 mediated ALS**
Muscular Dystrophy Association/Universtiy of Washington Subcontract
Co-PI (\$190,397)
The main study goal was to complete rodent and non-rodent toxicology studies needed to support the phase 1 study in people. Support for part of the phase 1 trial included.
- 2007-2011 **Phase II trial of R-pramepexole in ALS**
Knopp Neuroscience (Industry)
Steering Committee Member, Overall, PI and Site PI (\$795,700)
The main goal was to determine the safety and pharmacokinetics of three dosages of R-pramepexole in ALS. MGH is the Coordination and Data Management Center for the

- open label component of study.
- 2007-2011 **Metabolic Signatures in ALS**
 The Amyotrophic Lateral Sclerosis Association (Foundation)
 Overall, PI and Coordination and Data Management Center (\$445,127)
 This is a 30 center study to create a biobank with blood and cerebrospinal fluid from 600 well characterized people with ALS, disease mimics and health controls. Samples are shared with investigators studying ALS.
- 2007-2012 **Clinical Trial Methods Course**
 NINDS HHSN271200700018C
 Co-PI (\$62,171)
 The NINDS awarded this contract to conduct a yearly training course for neurologists (fellows and junior faculty) interested in careers in clinical trial research.
- 2007-2019 **TREAT ALS NEALS Clinical Trial Network**
 The Amyotrophic Lateral Sclerosis Association (Foundation)
 PI (\$2,397,993)
 The main goal is to provide infrastructure support to facilitate and expedite clinical trials Through the Northeast ALS Consortium (NEALS) (2016-2019, \$809,731)
 Specific clinical trial management support to Phase II Clinical Trial to Evaluate Safety, Tolerability, and Biomarker Engagement of AMX0035 in Patients with ALS (Study PI Paganoni, S) (2016-2019, \$854,080)
- 2008-2009 **Lithium in ALS**
 The Amyotrophic Lateral Sclerosis Association (Foundation)
 PI (\$153,662)
 This was an investigator initiated clinical trial (36 sites) to determine the efficacy of lithium in ALS.
- 2008-2009 **Coordinating Center: A Multicenter, Double-Blind, Placebo-Controlled, Study to Investigate the Safety and Efficacy of Lithium in Combination with Riluzole in Volunteers with Amyotrophic Lateral Sclerosis (ALS)**
 National Institutes of Health NINDS U01 NS049640-04S1
 PI (\$891,845)
 This was an investigator initiated clinical trial (36 sites) to determine the efficacy of lithium in ALS.
- 2008-2009 **A Double Blind, Randomized, Placebo-Controlled, Multicenter Study to Assess the Safety and Efficacy, and to Determine the Pharmacokinetics of Two Doses of AVP-923 (Dextromethorphan/Quinidine) in the Treatment of Pseudobulbar Affect (PBA) in Patients with Amyotrophic Lateral Sclerosis and Multiple Sclerosis**
 Avanir (Industry)
 Site PI (\$19,719)
 This was a pivotal, industry led phase 3 trial of a symptomatic treatment for PBA in ALS. The trial was positive and led to FDA approval.
- 2008-2010 **Trial Design Assistance Involving the use of ACE-031**
 Acceleron Pharma, Inc
 PI (up to \$16,000)
 The goal of this project was to design a Phase I and II study to test ACE-031, a drug that increases muscle bulk in people with ALS.
- 2008-2010 **A Multinational, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel Group Study to Assess the Efficacy, Tolerability and Safety of Talampanel in Subjects with Amyotrophic Lateral Sclerosis (ALS)**
 Teva (Industry)

- Site PI (\$165,025)
MGH was a site in this phase 3 industry led trial of talampanel, an AMPA glutamate receptor, in people with ALS.
- 2008-2012 **Coordination Center for trial of antisense oligonucleotide therapy for SOD1 mediated ALS**
ISIS (Industry)
PI, Coordination and Data Management Center (\$2,290,507)
This is a joint academic and industry led phase, single dose study of a gene therapy approach for SOD1 ALS. It is the first use of an antisense oligonucleotide treatment in a neurological Disorder.
- 2008-2011 **ALS Clinical Research Network**
Muscular Dystrophy Association (Foundation)
PI (\$300,000)
The MDA provides infrastructure support to five network centers, including MGH, to perform collaborative clinical research projects.
- 2008-2016 **MDA Clinic Director**
PI (100,800/year)
The MDA provides grant support on a yearly basis for our ALS multidisciplinary clinic.
- 2009-2011 **A phase II, double blind, randomized, placebo-controlled, three-way crossover, pharmaco-kinetic and pharmacodynamic study of CK-2017357 in patients with ALS**
Cytokinetics, Inc.
Coordination Center PI, Steering Committee Member (\$459,623)
We conducted a phase 1, single dose, study of CK-2017357, a troponin skeletal muscle activator in people with ALS. MGH was the Coordination Center for the study and a site.
- 2009-2013 **Multi-Center Validation of Biomarkers for Mother Neuron Disease**
University of Pittsburgh Subcontract – ARRA Funded RC1NS068179
Co-PI; Coordination and Data Management Center (\$384,779)
The goal of this collaborative study was to collect and test cerebrospinal fluid and blood proteomic biomarkers as possible biomarkers in ALS.
- 2009-2013 **Trial of High Fat/High Calorie Diet Versus Optimal Calorie Replacement in ALS**
MDA
PI (\$450,233)
The goal of this phase 2, academic led study is to determine the safety and tolerability of high fat, high calorie diet in people with ALS who have gastrostomy tubes.
- 2009-2011 **Generation and Characterization of ALS IPS Cells**
Johns Hopkins University Subcontract – ARRA Funded RC1NS069395
Data Management PI \$25,741
This resource grant is designed to collect skin biopsies for iPS cell lines from patients with genetic mutations associated with familial ALS. Clinical data was managed by MGH.
- 2009-2017 **Phase 1 Open-Label First in Humans, Feasibility and Safety Study of Human Spinal Cord Derived Neural Stem Cell**
Neuralstem, Inc.
Coordination and Data Management Center PI (\$964,493)
This is the first FDA approved phase 1 clinical trial of stem cells in people with ALS. MGH is the Coordination and Data Management center for this joint academic and industry led study. Surgery and cell implantation occurs at Emory.
- 2010-2016 **Specialized Program of Translational Research in Acute Stroke (SPOTRIAS)**
Data Management Core

- NINDS, P50 NS051343
Co-PI, Biostatistics and Data Management Core (\$1,280,774)
The main goal is to provide data management support for all funded SPOTRIAS stroke project at MGH.
- 2011-2013 **Phase 2 Randomized, Double-Blind, Placebo-Controlled, Multicenter Study of NP001 in Subjects with Amyotrophic Lateral Sclerosis (ALS)**
Neuraltus Pharmaceuticals, Inc
PI (\$275,456)
The primary objective is to evaluate the efficacy of NP001 in subjects with ALS using the mean change in slope of ALSFRS-R.
- 2011-2014 **Clinical Research Support Agreement – EMPOWER**
Biogen IDEC, Inc
PI (\$87,136)
The goal of this project is to provide services for Biogen Idec in connection with the EMPOWER Trial.
- 2011-2015 **A Multicenter Study for the Discovery and Validation of ALS Biomarkers**
ALS Therapy Alliance, Inc.
PI (\$1,095,961)
The aims of the proposed study are to create a biofluid repository of plasma and CSF collected from patients with amyotrophic lateral sclerosis (ALS) serially every 4-6 months for at least 2 years, and to use these longitudinal samples to explore novel biomarkers of ALS diagnosis and progression.
- 2011-2015 **Creation of the Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) Database study**
Prize4life ATA
Co-PI (\$197,119)
The primary objective is to aid creation of the pooled resource open-access ALS Clinical Trials database.
- 2011-2015 **Phase II/III Study of Arimoclomol in the Treatment of SOD1 Positive Familial Clinical**
University of Miami, R01FD003517
Co-PI (\$81,372)
The primary objective is to study the effects of Arimoclomol in the treatment of SOD1 positive.
- 2011-2016 **Trial of Resistance and Endurance Exercise in ALS**
John's Hopkins University
PI (\$43,862)
The goal of the trial is to evaluate resistance and endurance exercises in ALS patients.
- 2012-2014 **Validation of a self-report scale for the assessment of bulbar function**
ALS Association 2106B
Co-Investigator (\$269,981)
Develop outcome measures for ALS
- 2012-2016 **NeuroNEXT-SMA Biomarkers in the Immediate Post-Natal Period of Development**
NINDS U01NS079163
Clinical Coordination Center PI (\$934,892)
The primary objective of this study will focus upon the natural history of SMA biomarkers in the immediate postnatal period of development.
- 2012-2017 **Environmental Risk Factors for ALS in a Representative Sample of the US**
President and Fellows of Harvard, R56NS08210

- PI (\$33,870)
A study of possible environmental risk factors for ALS.
- 2013-2016 **Noninvasive assessment of neuromuscular disease using electrical impedance**
R44 NS070385
Co-Investigator (\$378,095)
NCRI will provide support for project and data management, and biostatistical support.
- 2013-2017 **Multi-Center, Randomized Controlled Study of the NeuRX Diaphragm Pacing System in Participants with ALS**
ALS Association (SUNY)
PI of Coordination Center (\$450,000)
The NCRI Coordination Center is responsible for the overall project management of all study activities.
- 2013-2017 **Multi-Center, Randomized Controlled Study of the NeuRX Diaphragm Pacing System in Participants with ALS**
Synapse (SUNY)
PI of Coordination Center (\$912,168)
The NCRI Coordination Center is responsible for the overall project management of all study activities.
- 2013-2018 **NeuroNEXT-Ibudilast Phase II Trial in Progressive MS**
Cleveland Clinic (Fox, R PI); U01 NS082329
Clinical Coordination Center PI (\$5,526,813)
This study proposes to both test the activity of a potential neuroprotection therapy - ibudilast -in progressive MS and test several candidate imaging measures for their utility in future studies of neuroprotection in progressive MS.
- 2013-2018 **NeuroNEXT-Rituximab in the management of refractory autoimmune MG**
Yale University (Nowak, R PI); U01NS084495
Clinical Coordination Center PI (\$2,641,456)
The primary aim of this study is to determine whether rituximab is safe and shows sufficient promise as a steroid sparing therapeutic for MG to warrant further study in a phase III efficacy trial.
- 2014-2016 **NeuroNEXT-Rituximab in the management of refractory autoimmune MG**
Yale University (Nowak, R PI); U01NS084495
Clinical Coordination Center PI (\$2,641,456)
The primary aim of this study is to determine whether rituximab is safe and shows sufficient promise as a steroid sparing therapeutic for MG to warrant further study in a phase III efficacy trial.
- 2015-2017 **Proof-of-concept study of RNS60 in ALS** (PI Paganoni, S, MGH)
Philanthropy
Co-PI (\$694,000)
The objective of this proof-of-concept study is to measure the effects of RNS60 on reducing brain microglial activation measured by [11C]-PBR28-PET and its effects on peripheral immune markers in 15 people with Amyotrophic Lateral Sclerosis (ALS). We will also assess safety and effects on clinical measures of illness.
- 2014-2018 **A Phase II Randomized Placebo-controlled Trial of Tocilizumab in ALS** (PI Ladha, S, Barrows Neurological Institute)
The Amyotrophic Lateral Sclerosis Association (Foundation); 15-TACL-187 (\$154,508)
Philanthropy (\$500,000)
Co-PI Coordination Center

The primary aims of this project are to determine the safety and tolerability of up to 8mg/kg per month of tocilizumab in ALS and to demonstrate the attenuation of pro-inflammatory pathways that play a role in motor neuron death in ALS. Secondary aims are to demonstrate the pharmacokinetics of the study drug in blood and CSF, as well as to preliminarily assess for an efficacy signal.

- 2014-2018 **A Proof of Concept Study to Evaluate the Safety, Tolerability and Efficacy of SYN120, a dual 5-HT6/5-HT2A Antagonist, in Parkinson's Disease Dementia**
Biotie Therapies Inc (Michael J Fox Foundation)
Coordination Center PI:(\$3,494,000)
Evaluate the safety, tolerability and efficacy of SYN120 in PDD patients.
- 2014-2018 **NeuroNEXT Trial: ZZ-3K3A-201: Safety Evaluation of 3K3A-APC in Ischemic Stroke**
Cedars-Sinai Research Institute (Lyden, P PI), U01NS088312
Clinical Coordination Center Director (\$3,437,014)
An evaluation of the safety of 3K3A-APC in Ischemic Stroke.
- 2014-2018 **Autologous Regulatory T Lymphocyte Therapy in ALS**
ALS Finding a Cure(Foundation)
Coordination Center PI: Cudkowicz (\$180,000)
The primary objective of the first stage of the pilot study is to determine the safety and tolerability of infusions of autologous Treg cells in 2 patients with ALS. The secondary objective will be to determine whether the administration of autologous Treg cells with or without concomitant subcutaneous IL-2 therapy increases the number of Treg cells in peripheral blood in ALS patients.
- 2014-2018 **Phase 2 pharmacodynamic study of retigabine on neuronal excitability in ALS Subjects**
ALS Association TACL-215
Coordination Center PI (Study PI: Wainger, B)
The major goals of this project are to measure effect of retigabine on motor neuron excitability, determine safety, dosing and pharmacokinetics of retigabine in ALS subjects and obtain skin biopsies for iPSC motor neuron differentiation and *in vitro* motor neuron physiological profiling
- 2014-2019 **Inflammatory Biomarkers, Stem Cells, and DNA in People with ALS (ENROLL)**
ALS Association / ALS Finding a Cure (Atassi)
Co-Investigator
The major goal of this project is a study in the inflammatory Biomarkers, stems cells, and DNA in people with ALS. This project aspires to identify biomarkers of ALS that will give us clues to disease mechanisms and help us identify targets for ALS therapy development.
- 2014-2020 **Safety and Tolerability Study of AAV10-mediated, miRNA-based silencing of the SOD1 gene in Patients with SOD1-mediated Familial Amyotrophic Lateral Sclerosis**
ALS Finding a Cure(Foundation)
Coordination Center PI (\$45,600)
The primary objective of the study is to determine the safety and tolerability of this therapy.
- 2015-2017 **Proof-of-concept study of RNS60 in ALS** (PI Paganoni, S, MGH)
Philanthropy
Co-PI (\$694,000)
The objective of this proof-of-concept study is to measure the effects of RNS60 on reducing brain microglial activation measured by [11C]-PBR28-PET and its effects on

- peripheral immune markers in 15 people with Amyotrophic Lateral Sclerosis (ALS). We will also assess safety and effects on clinical measures of illness.
- 2015-2018 **Effects of mexiletine on cortical hyperexcitability in amyotrophic lateral sclerosis**
 (PI: M. Weiss, University of Washington) ALS Association 16-TALS-298
 Philanthropy (\$500,000)
 Clinical Coordination Center PI
 The objective of this phase 2 study is to determine the effects of mexiletine on cortical hyperexcitability in ALS participants using transcranial magnetic stimulation. Stem cells secreting GDNF for the treatment of ALS
- 2015-2018 **NeuroNEXT-Tolerability of SRX246 in Huntington's Disease**
 Azevan/MGH (Goldstein, M PI) 4U44NS090616
 Clinical Coordination Center PI (\$2,940,977)
 The study goal is the test the effect of SRX246 on irritability in people with Huntington's Disease.
- 2015-2018 **Phase 3 trial of inosine for Parkinson's Disease**
 U01NS090259 (MGH Schwarzcchild, M PI)
 Co-PI (Clinical Coordination Center)
 The major goal of this project is to determine whether inosine dosed to raise serum urate levels slows clinical progression in early Parkinson's Disease.
- 2015-2020 **DR2A-05320; Cedars Sinai Research Institute (CIRM grant)**
 Sub-Award PI data coordinating center (PI Svendsen, C)
 The primary goal of this project is to help with the study of human neural progenitor cells expressing glial cell line derived neurotrophic factor (CNS10-NPCGDNF) for the treatment of ALS.
- 2015-2021 **ANSWER ALS (PI): Rothstein/Berry**
 ALS Finding a Cure (Foundation)
 Co-Director (\$546,713 total direct costs)
 ANSWER ALS is designed to generate iPS brain cells from a large population of ALS patients to comprehensively evaluate their genetic and metabolic profiles and perform comprehensive longitudinal clinical observations.
- 2016-2018 **Massachusetts ALS Partnership Program (MAPP) (Co-Directors: Berry and Atassi)**
 ALS Association, ALS Finding a Cure and ALS One (Foundations)
 Investigator (\$1,800,000)
 The primary goals of this program are to: 1- Build an efficient regional ALS clinical trials network to translate promising lab findings into treatments for people with ALS. 2- Build a translational pipeline that includes the development of new lab tests and imaging tools to efficiently test treatment effects in ALS patients, and the expansion of a clinical trial of a medication (Ibudilast) that targets inflammation as one of the proposed ALS mechanisms. 3- Move the multidisciplinary care at MGH beyond the walls of the clinic to improve access and integrate with Compassionate Care ALS (CCALS) and other home care providers using novel technology and nursing resources.
- 2016-2021 **NeuroNEXT- Cytochrome C Oxidase: Biomarker in Newly Diagnosed Glioblastoma Multiforme**
 University of Iowa (Griguer, C PI) U01NS093663
 Clinical Coordination Center PI (\$863,580 total direct costs)
 The primary objective of this study is to evaluate the hypothesis that overall survival time of participants with newly diagnosed GBM tumors are a function of CcO enzymatic activity in the tumor.
- 2016-2021 **NeuroNEXT-Effects of AFQ056 for Language Learning in Children with FXS**

Rush University Medical Center (Berry-Kravis, E PI) U01NS096767
Clinical Coordination Center PI (\$5,603,338 total direct costs)

The primary objective of this study is to evaluate the effect on language in young children with FXS treated with AFQ056 in combination with an intensive, standardized parent implemented language intervention.

2017-2021 **NeuroNEXT- Topiramate as a Disease Altering Therapy for CSPN**

Virginia Commonwealth University (Smith, G PI) U01NS095388

Clinical Coordination Center PI (\$3,477,385 total direct costs)

The primary objective is to determine if topiramate slows progression of cryptogenic sensory polyneuropathy (CSPN).

2017-2022 **Collaboration to Develop an ALS Clinic and Clinical Trials in ALS**

Holy Cross Hospital

PI (\$1,352,642 total direct costs)

The primary goal is to provide consultation, clinical assistance and professional support in the development and implementation of research and clinical activities dedicated to the study and treatment of patients with neurological illnesses, disorders and conditions at the Smith ALS Clinic at Holy Cross Hospital.

2018-2021 **Expanded Population of Regulatory T-Cells (EPAR T-Cells) for People with Amyotrophic Lateral Sclerosis (PALS)**

ALS Association | ALS Finding a Cure

Co-Investigator (\$750,000 total direct costs)

The primary aims of this study are to evaluate the biological activity of IV infusion of an expanded population of autologous regulatory (EPAR T-Cells) and low-dose IL-2 in people with ALS by evaluating Treg number and suppressive function; to evaluate the safety and tolerability of IV infusion of an expanded population of autologous regulatory T-cells (EPAR T-Cells) and low-dose IL-2 in people with ALS; and to characterize the effects of EPAR T-Cells and low-dose IL-2 on clinical outcome measures of ALS including Appel ALS Scale (AALS) and ALS Functional Rating Scale-Revised (ALSFRS-R) scores.

2020-2022 **ALSA Clinical Trial Support**

ALS Association

PI (\$352,416 total direct costs)

The primary objective of this study is to 1) Support Therapeutic Pipeline Development 2) Develop and Promote Quality Clinical Trials and 3) Enhance Clinical Trial Access and Participant Recruitment, Retention, and Experience.

Current

2011-2023 **NeuroNEXT Clinical Coordination Center**

NINDS, U01NS077179

PI (\$7,499,997 total direct costs)

The primary object is to act as the Clinical Coordinating Center for a NINDS sponsored 25 site Network, to conduct efficient, high impact, and high-quality studies. Additional objectives include education and training of clinical investigators and staff at each site.

2017-2023 **NeuroNEXT - A Randomized, Double-Blind, Placebo-Controlled, Multi-Center Study to Evaluate the Efficacy of ManNAc in Subjects with GNE Myopathy**

Brigham & Women's Hospital (Amato & Carrillo PI) U01AR070498

Clinical Coordination Center PI (\$2,531,682 total direct costs)

The primary objective of this study is to evaluate the efficacy of ManNAc in participants with GNE myopathy as measured by the ability to slow the progression of muscle strength decline compared to placebo. This study is a collaboration between NINDS and NIAMS.

- 2019-2023 **NeuroNEXT - A Dose Selection Trial of Light Therapy for Impaired Sleep in Parkinson's Disease**
 MGH (Videnovic PI) U01NS114001
 Clinical Coordination Center PI (\$2,644,099 total direct costs)
 The primary objective of this study is to examine the optimal dose frequency of LT to improve sleep in PD and carry forward in future clinical trials.
- 2019-2024 **HEALEY ALS Platform Trial | Accelerating the Path to Effective Treatments for ALS**
 ALS Association, PI (\$2,608,696 total direct costs) | Jan 2020 to Dec 2022
 Finding a Cure, PI (\$1,391,304 total direct costs) | Apr 2020 to Mar 2022
 Muscular Dystrophy Association, Co-I (\$454,545 total direct costs) | Apr 2021 to Mar 2024
 M. Blank Family Foundation, PI (\$500,000 total direct costs) | Dec 2019 to Nov 2021
 UCB/Ra Pharmaceuticals, Inc., PI (\$9,497,075 total direct costs) | Oct 2019 to Apr 2023
 Biohaven Pharmaceuticals, Inc., PI (\$9,933,104 total direct costs) | Oct 2019 to Apr 2023
 Clene Nanomedicine, Inc., PI (\$6,164,018 total direct costs) | Oct 2019 to Dec 2021
 Prilenia Neurotherapeutics Ltd., PI (\$9,461,538 total direct costs) | Nov 2019 to Jun 2022
 Seelos Therapeutics, Inc., PI (\$9,085,372 total direct costs) | Jan 2021 to Jun 2023
 Revealisio, Inc., PI (\$486,055 total direct costs) | Jan to May 2021
 The primary objective of this phase 2/3 perpetual multi-center, randomized, placebo-controlled, clinical trial is to evaluate the safety and efficacy of multiple investigational products for the treatment of ALS. Several investigational products will be tested in parallel and sequentially, using a shared infrastructure, common data and sample collection processes, and central governance.

Report of Local Teaching and Training

Teaching of Students in Courses

2000	Epidemiology 208 Literature Critique HSPH Students	HSPH Guest speaker three hours
2004	EPI 284c Epidemiology of Neurological Diseases HSPH Students	HSPH Guest speaker five hours
2001	HST 160 Molecular Biology and Genetics in Modern Medicine 1 st year medical students	HST Supervised 3 students ten hours
2006, 2010	HST 11 The Art and Science of Medicine MIT undergraduate students	HST Guest speaker 20 students six hours
2008, 2009, 2011, 2012	HST 130 Introduction to Neuroscience 2 nd year medical students	HST Guest speaker Three hours once per year
2019	HST MD Introduction to the Profession for Physician-Scientists	HST MD

Formal Teaching of Residents, Clinical Fellows and Research Fellows (post-docs)

2001	Neuroepidemiology Neurology residents	MGH and BWH One hour lecture per month for a year
2008-2010	Neuroscience Seminar Series Neurology residents	MGH and BWH One hour lecture

2009	Drug development in ALS Clinical research fellows	BIDMC, Clinical Investigator Training Program Two and half hour's lecture
2009-2011	Becoming a Principal Investigator for multi-center clinical trials Clinical research fellows	MGH Five hours lecture
2011	Resident Neuroscience Journal Club Neurology residents and fellows	One and half hours
2012-	Chief Resident Rounds MGB residents and fellows	2 hours/month

Clinical Supervisory and Training Responsibilities

1996-	Neurology Ambulatory Care Clinic Preceptor MGH	Four to five half sessions per week
1996-2007	Neurology Inpatient Unit Attending physician MGH	Four weeks per year
2005-	Neuromuscular Ambulatory Care Clinic Preceptor MGH	One half session per week
2007-2011	Neurology Inpatient Unit Attending physician MGH	Two weeks per year

Laboratory and Other Research Supervisory and Training Responsibilities

2008-	Supervision of research fellow/ALS	Daily mentorship for twelve months of one-two non-US trained MD research fellows
2009-2011	Supervision of Spaulding Rehabilitation Hospital Resident in Research	Monthly meetings

Formally Supervised Trainees

2001-2008	Mudasir Qureshi, MD/Pediatric Resident, Texas Tech University Health Science. Published six first author papers, one review and one meeting proceedings paper.
2002-2004	Bryan Traynor, MD, MMSc, MRCPI/ Chief Neuromuscular Diseases Research Group NIA, NIH. Clinical Investigator Training Program fellowship awardee; Published two first author papers; faculty position at NIH and adjunct faculty position at John Hopkins University.
2004-2005	Rabia Choudry, MD/Staff Neurologist, Baylor University Medical Center. Published up-to-date manuscript on ALS. Developed ALS center in Texas.
2005-2007	Allitia Dibernardo, MD/Medical Director Johnson and Johnson. Published important pharmacodynamic study of Creatine in ALS. Leading development of new treatments for neuromuscular disorders. Senior Medical Director of US Affairs at Novartis Pharmaceuticals Corporation.
2006-2009	Anne Marie Wills MD MPH/Assistant Professor of Neurology, HMS. 2006 American Academy of Neurology/ALS Association Clinician Scientist Development Awardee, 2009 Muscular Dystrophy Association Neuromuscular Clinical Research Fellowships. Published two important papers on genetics of ALS.

- 2006-2009 Leigh Hochberg, MD PhD/Visiting Associate Professor of Neurology, HMS. Professor Brown University. Multiple published articles on use of brain chips for communication for patients with ALS and spinal cord injury. Invited plenary speaker at American Academy of Neurology.
- 2007-2010 Swati Aggarwal, MD/ 2009 American Academy of Neurology, ALS Association Clinician Scientist Development Awardee. Published pivotal clinical trial paper in Lancet Neurology.
- 2008-2019 Nazem Atassi, MD MSc/Associate Professor of Neurology, HMS. Clinical Scholars Program graduate, T32 award, Muscular Dystrophy Association Clinical Research Fellowship. Biogen-Idec ABY Fellowship, K23. VP Sanofi
- 2010- James Berry, MD MSc/Associate Professor of Neurology, MGH. T32 award, Muscular Dystrophy Association Clinical Research Fellowship, AAN Rick Olney ALS Clinical Research Fellowship, Neurology Department Merit Awardee 2011.
- 2010 Steve Han, MD PhD/Assistant of Neurology, HMS, Medical Director Takeda, Clinical Investigator Training Program Fellow, 2011 American Academy of Neurology/ALS Association Clinician Scientist Development Awardee, KL2 MeRIT Awardee 2012.
- 2010- Sabrina Paganoni, MD PhD/Assistant Professor of Physical Medicine and Rehabilitation, HMS, K12 Rehabilitation Medicine Scientist Training Awardee.
- 2012-2016 Haruhiko Banno, MD PhD/ Clinical Research Fellow, MGH.
- 2012-2016 Elena Ratti, MD/ Instructor, HMS, Biogen-Idec ABY Fellowship, Biogen Associate Medical Director
- 2017-2021 Sheena Chew, MD/Instructor, HMS, Biogen ABY Fellowship; Currently Biogen MD
- 2014- Katie Nicholson, MD/Instructor, HMS, Biogen ABY Fellowship, Sanofi Clinical Research Director
- 2016- Suma Babu MD/Assistant Professor Neurology, HMS, AAN Clinical Research Fellowship
- 2017-2021 Sheena Chew, MD/Instructor, HMS, Biogen ABY Fellowship; Currently Biogen Medical Director in Clinical Development

Formal Teaching of Peers (e.g., CME and other continuing education courses)

- | | | |
|-------------|----------------------------------------------|----------------------|
| 1994, 1996, | Intensive Review of Neurology | Lecture |
| 1997, 2008 | Neurology, Harvard Medical School | Boston |
| 1999, 2001 | Electromyography and Clinical | Lecture |
| 2009, 2010 | Neurophysiology | Boston |
| | Harvard Medical School | |
| 2011 | Principles and Practice of Clinical Research | Lecture |
| | Harvard Medical School | Boston and web-based |
| 2011 | Managing Your Team, Harvard Catalyst | Invited Speaker |
| | | Boston |
| 2012 | The Study Team, NEALS | Invited Speaker |
| | New Principal Investigator Training | Charlestown |

Local Invited Presentations

- 2001 Remacemide and Coenzyme Q10 Therapy in Huntington's Disease/ Invited Speaker
General Clinical Research Center
- 2002 Results of CARE-HD trial/ Speaker Series
Department of Neurology, MGH

2002 Genetics of ALS/ Speaker Series
Massachusetts General Physician Organization

2002 Translational research in ALS/ Grand Rounds
Department of Neurology, MGH

2003 Harvard Clinical Trial Service/ Update
Harvard Center Neurodegeneration and Repair

2004 How to fund clinical trials/ Lunchtime Speaker Series
Department of Neurology, MGH

2004 How do we find treatments for ALS/ Grand Rounds
Department of Neurology, MGH

2008 ALS a Brave New World/ Speaker
Harvard Medical School Alumni Week

2008 Translation towards effective ALS therapy/ Update
MGH Chief's council

2004 How to fund clinical trials/ Lunchtime Speaker Series
Department of Neurology, MGH

2008 Lost and found in translation: ALS/ Update Neurology Retreat
Department of Neurology, MGH

2010, 2011 IND/IDEs – Beyond the basic investigational product applications/ Speaker Series
Clinical Research Education Unit, MGH

2010, 2011 Sponsoring and Managing Multi-Center Clinical Trials/ Speaker Series
Clinical Research Education Unit, MGH/ Speaker

2011 MGH Bicentennial Celebration

2012 ALS: New Genes, New Treatments and Renewed Hope/ Grand Rounds
Brigham and Women's Hospital

2012 Drugs, gene therapy, stem cells: Therapeutic revolution in ALS/ Invited Plenary Speaker
New England Science Symposium, Harvard Medical School

2012 ALS Care: Tackling Symptoms and Disease Progression/Palliative Care/ Grand Rounds
MGH

2012 From Chemical Engineering to Clinical Department Chair: Lessons learned as a Woman
in Academic Medicine/ Invited Speaker
Office for Women's Careers, MGH

2014 Update on ALS Clinical Trials/ Grand Rounds
Newton-Wellesley Hospital, Newton, MA

2015 ALS Research and Treatment Advancements/ Spring HST Dinner Seminar/ Invited
Speaker
Harvard Club of Boston

2015 MGH Clinical Research Day: How to think about innovation and translation at MGH/
Invited Panel Speaker
MGH

2018 Developing Effective ALS Therapies: Lessons From Recent Clinical Trials/ Invited
Speaker
Cell Press LabLinks

2018 C/T Academy Research Day: Graduation Address/ Invited Keynote Speaker
The Harvard Clinical and Translational Science Center

2020 New Advances in ALS, ECOR presentation with Dr Clotilde Lagierre Tourenne

2021 Life Post Residency, Choosing Best Position & Academic Medicine / Invited Speaker
Neurology Department, Massachusetts General Hospital, Boston, MA

2022 Medical Exchange Club, ALS - New Therapies and Trial Approaches /

- 2022 Invited Speaker. Virtual. Harvard Medical School, Boston, MA
Target ALS Precompetitive Biomarker Consortium. Platform Trials Update. Invited Speaker. In-Person Boston, MA
- 2022 World Medical Innovation Forum. The Patient Experience / Invited Speaker
In-person Westin Copley, Boston, MA
- 2022 BWH Tyler Lecture Grand Rounds / Invited Speaker/Virtual
Brigham and Women's Hospital
- 2022 MGH Tyler Lecture Grand Rounds / Invited Speaker/Virtual
Massachusetts General Hospital

Report of Regional, and International Invited Teaching and Presentations

Invited Presentations and Courses

Regional

- 1996, 2005 ALS Clinical Trials/ Grand Rounds
Beverly Hospital, Beverly, MA
- 2002 Update on mouse and human studies in ALS/ Invited Speaker
MDA Regional Science Meeting, Charlestown, MA
- 2003 Experimental therapies in ALS/ Grand Rounds
Boston University School of Medicine, Boston, MA
- 2004 Novel Therapies for ALS/ Invited Speaker
University of Massachusetts, Worcester, MA
- 2006 How to launch a clinical trial/ Invited Speaker
Northeast ALS consortium 5th Annual Meeting, Boston, MA
- 2007 Safety and dose escalating study of oral sodium phenylbutrate in subjects with ALS
(abstract) Treatment of ALS / Course Lecture Neuromuscular Therapy/ Invited Speaker
American Academy of Neurology 59th Annual meeting, Boston, MA
- 2008 ALS clinical trials; designs and challenges/ Invited Speaker and Course organizer
China ALS Training Course, Boston, MA
- 2009 Update on ALS Therapeutics/ Grand Rounds
Tufts Medical School, Boston, MA
- 2011 State of Therapeutic Development in ALS/
Massachusetts Biotechnology Society, Cambridge, MA (Prize4Life)
- 2011 ALS Innovation Award Meeting
Cambridge, MA
- 2012 New England MDA Clinic Directors' Meeting/
Charlestown, MA
- 2013 ALS: New Directors/
ALS Research Day, Lexington, MA (Shire Pharmaceuticals)
- 2014 ALS Update/ Invited Speaker
Head Health Initiative. NFL Headquarters, New York, NY
- 2015 ALS Therapeutic Advances/ Grand Rounds
New York University, New York, NY
- 2015 Clinical Demonstrations/ Moderator
World Medical Innovation Forum, Boston, MA
- 2015 Neurocare, Neuromarkets, and Neuro Challenges/ Panelist
World Medical Innovation Forum, Boston, MA
- 2015 Solving ALS: Lessons from Multiple Disciplines/ Invited Speaker
10th Annual HSCI Malkin Retreat, Cambridge, MA

- 2020 Career Advice: Life After Residency. Finding a Job and Starting a Career in Academic Medicine/Invited Speaker
Massachusetts General Hospital Resident's Conference. Virtual Lecture, Boston, MA
- 2020 2020 ALS ONE Research Symposium: 2020 View of ALS Therapeutics.
ALS ONE, MA

National

- 1996 Intrathecal SOD1 as a treatment for ALS/ Invited Speaker
Pharmaceutical Research and Manufacturers of America Foundation Meeting, Washington DC
- 1997 Riluzole therapy in Huntington's Disease/ Invited Speaker
Huntington's Disease Study Group Annual Meeting, Santa Fe, NM
- 1998 Superoxide dismutase 1 and ALS/ Grand Rounds
Yale University, Department of Neurology, New Haven, CT
- 1998 Riluzole therapy in patients with Huntington's disease/ Invited Talk
American Academy of Neurology 50th Annual Meeting
- 1999 Recent advances in the treatment of ALS/ Invited Speaker
Columbia University Sergievsky Center, New York, NY
- 1999 Effects of altered levels of GSHPx on disease phenotype in the ALS mouse model/
Invited Speaker
The Banbury Center, ALS Association Scientific Workshop, Cold Spring Harbor, NY
- 2000 Therapeutic advances in ALS/ Invited Speaker
Medtronic Inc. CNS Infusion Board Meeting (Medtronic)
- 2000 Clinical trial of Minocycline in HD/ Invited Speaker
Huntington's Disease Study Group Annual Meeting, La Jolla, CA
- 2000 Will Topiramate slow disease progression in ALS/ Invited Speaker
Epilepsy Conference, New Orleans, LA (Ortho-McNeil)
- 2000 Clinical Trial of Celebrex in ALS/ Invited Speaker
Pfizer Pharmacia New Initiatives meeting, Philadelphia, PA (Pfizer Pharmacia)
- 2001 Study design methodology in ALS. Experience of the Northeast ALS Consortium/
Invited Speaker
ALS Association Drug Company Working Group Meeting, Denver, CO
- 2001 Therapeutic advances in ALS/ Grand Rounds
State University of New York, Upstate Medical University, Syracuse, NY
- 2002, 2006 Experimental therapeutics in ALS/ Invited Speaker
ALS Association Clinical Conference/Leadership Conference, Washington DC
- 2002 Clinical trial of Topiramate in ALS: Risk for abnormal clotting events/ Invited Speaker
Ortho-McNeil experts panel on ALS and deep venous thrombosis (Ortho-McNeil)
- 2003 Clinical outcome measures in ALS/ Invited Speaker
Tarrytown ALS Research Meeting, Tarrytown, NY
- 2003 ALS: From the mouse colony, to the bedside and back again/ Plenary Invited Speaker
American Neurological Association, San Francisco, CA
- 2004 New drug therapies in ALS/ Invited Speaker and Participant
NINDS/ALS Association Drug Discovery Workshop, New York City, NY
- 2004 ALS Diagnosis and Management, ALS Lessons from genetics and new treatment initiatives, and Huntington's Disease, a double blind futility study of Minocycline in HD
Fifth Annual Jules Schwartz ALS Lecture/ Visiting Professor
University of Rochester, Rochester, NY
- 2004 Celebrex clinical trial/ Invited Speaker
Jazz Pharmaceutical ALS Advisory Board Meeting, Scottsdale, AZ

- 2004 Outcome measures in ALS/ Invited Plenary Speaker
American Academy of Electromyographers 51st Annual Meeting, Savannah, GA
- 2004 Challenges of clinical investigation/ Invited Speaker
Huntington's Disease Study Group 12th Annual Meeting, St Louis, MO
- 2004 Novel therapies in ALS/ Grand Rounds
Brown University, Department of Neurology, Providence, RI
- 2005 New approaches to ALS therapies/ Invited Speaker
Packard Center Board Meeting, New York City, NY
- 2005 Clinical study designs in Huntington's disease/ Invited Speaker
HiQ Foundation Scientific Advisory Board Meeting, New York City, NY
- 2006 Therapeutic advances in ALS/ Grand Rounds
Cornell Medical School, New York City, NY
- 2006 Clinical trial design in ALS studies/ Invited Speaker
ALS Association Drug Company Working Group meeting, San Diego, CA
- 2006 Clinical Research Career Options/ Invited Speaker
American Academy of Neurology, San Diego, CA
- 2006 Neuromuscular Scientific Highlights/ Invited Presenter
American Academy of Neurology 58th Annual Meeting, San Diego, CA
- 2006 Northeast ALS Consortium clinical research initiatives/ Invited Speaker
Packard Center Annual Meeting, Baltimore, MD
- 2006 Challenges in biomarkers and trial designs for SMA and ALS/ Invited Speaker
Neurobiology of Disease Workshop, Motor Neuron Disease, Atlanta, GA
- 2007 ALS: causes and cures/ Grand Rounds
University of Pennsylvania, Philadelphia, PA
- 2007 ALS 2007: Symptomatic management and new therapeutic options/ Invited Speaker
Lunch and Learn Sessions, Web based conference (NINDS)
- 2008 Update on current and ongoing clinical trials/ Invited Speaker
Overview of clinical trials in ALS/ Invited Speaker
ALS Association TREAT ALS Conference, Tampa, FL
- 2008 ALS medicines and mechanisms/ Grand Rounds
Albert Einstein, New York City, NY
- 2008 ALS diagnosis, management and clinical trial designs/ Invited Speaker
KNOPP/Omnicare ALS orientation and training symposium (KNOPP)
- 2008 ALS Therapy Development: A Brave New World/ Invited Plenary Speaker
American Society for Experimental NeuroTherapeutics 10th Annual Meeting, Arlington, VA
- 2008 Northeast ALS Consortium clinical trial initiatives/ Invited Speaker
Packard Center Annual Meeting, Baltimore, MD
- 2008 TREAT ALS NEALS clinical trial network/ Invited Speaker
ALS Association Drug Company Working Group, Chicago, IL
- 2009 Clinical Trials Update: Ceftriaxone/ Invited Plenary Speaker
Muscular Dystrophy Association National Clinic Directors Conferences, Las Vegas, NV
- 2009 Lessons learned from past trials in ALS/ Invited Speaker
Muscular Dystrophy Association Phase 2 trial design workshop, Washington DC
- 2009 ALS treatment trials: bridge from science to cures/ Plenary Talk, Sheila Essey Award
Lecture
American Academy of Neurology, Seattle, WA
- 2009 ALS therapeutics: lessons learned and path forward/ Grand Rounds
Emory University, School of Medicine, Atlanta, GA

- 2009 Successful participation in multi-institutional studies.
Where do I go from here?/ Invited Speaker
American Neurological Association Summer Course in Clinical and Translational
Research Vail
- 2009 Phase 2 trial designs/ Invited Speaker and Course Co-Director
Clinical Trials Method Course in Neurology, Vail (NINDS)
- 2009 Adaptive Clinical Trials: Ceftriaxone in ALS a seamless adaptive design study/Invited
course faculty
Huntington's Disease Study Group Annual Meeting, Baltimore, MD
- 2010 ALS treatments symptomatic and neuroprotective/ Grand Rounds
Medical University of South Carolina, Charleston, SC
- 2010 A 12 week phase 2 study of the safety, tolerability and clinical effects of KNS760704 in
ALS/ Invited Speaker
10th Annual Robert Packard Center ALS Research Symposium, Baltimore, MD
- 2010 KNS760704 Part 2 Study Results/ Invited Speaker
Knopp Neuroscience Webcast
- 2011 EMPOWER Investigator Meeting; Principal Investigator
ALS clinical trial updates
Phase 2 Dexpramipexole results and Phase 3 design
Recruitment and Retention Strategies,
Atlanta and Amsterdam, GA
- 2011 Research Career Development Symposium: Transitioning from Residency to Research/
Invited Speaker
American Academy of Neurology 63rd Annual Meeting, Honolulu, HI
- 2011 ALS Therapies-The Road to a Cure/ Grand Rounds
University of Arkansas, Little Rock, AK
- 2011 Careers in Clinical Research/ Invited Speaker
University of Arkansas, Little Rock
- 2012 Clinical trial of Ceftriaxone in ALS: Final results/ Invited Speaker
International Motor Neuron Disease Symposium, Chicago, IL
- 2012 Fellowship Recipient Announcement/ Moderator
American Academy of Neurology 64th Annual Meeting, New Orleans, LA
- 2012 The Empower Study: Design, Methodology and Baseline Features of the First Phase III
Clinical Trial of Dexpramipexole for Patients with ALS/ Invited Speaker
American Academy of Neurology 64th Annual Meeting, New Orleans, LA
- 2012 ALS Therapy Development-Progress and Hope/ Invited Speaker
Amyotrophic Lateral Sclerosis: Trials, Treatments, and Tribulations Conference
Hospital For Special Care, New Britain, CT
- 2012 University of Pennsylvania/ Grand Rounds
Philadelphia, PA
- 2012 12th Annual Packard Center Symposium/ Invited Speaker
Ceftriaxone and Dexpramipexole Phase III ALS studies, Baltimore
- 2012 ALS Therapy Development-Progress In Therapy/ Invited Speaker
Yale School of Medicine, New Haven, CT
- 2012 ALS Therapeutic Advances/ Invited Speaker
Fifth Stanley H Appel Resident Education Distinguished Lecture, Methodist Hospital,
Houston, TX
- 2012 ALS Update on Therapy Development/ Invited Speaker
MDA South Regional Meeting, Houston, TX

- 2013 University of Pittsburgh Medical Center/ Grand Rounds
The Leonard Gerson Distinguished Scholar Lecture, Pittsburgh, PA
- 2013 19th William L. Halseth ALS Visiting Professor/ Grand Rounds
University of Colorado Anschutz Medical Campus, Denver, CO
- 2013 Clinical Trials Plenary Session/ Invited Speaker
American Academy of Neurology 65th Annual Meeting, San Diego, CA
- 2013 The 29th Irwin Levy Visiting Lectureship/ Grand Rounds
Washington University in St. Louis, St. Louis, MO
- 2013 5th Annual Translational and Clinical Research Course for Clinician-Scientists/
American Neurological Association
- 2013 Focus Group Leadership Meeting
Washington, DC (ALSA/NEALS)
- 2013 Leaders in the Field Presentation: SIG Meeting. Presymptomatic Therapy in ALS/
Invited Speaker
American Neurological Association, New Orleans, LA
- 2013 C9ID Meeting/ Invited Speaker
Norfolk, VA
- 2013 ALS Therapeutic Revolution-small molecules, gene, and stem therapy/ Petajan Lecture
Series
University of Utah, Salt Lake City, UT
- 2014 14th Annual Robert Packard Center for ALS Research Symposium
NEALS/ Update on enrollment, active NEALS studies and initiatives
Baltimore, MD
- 2014 Inaugural Resident/ Invited Grand Rounds Speaker
ALS: Changing Definition and Approach to Therapy Development
Department of Neurology, University of Washington, Seattle, WA
- 2014 Swedish Neuroscience Grand Rounds
ALS: Therapeutic: New Frontier
Seattle, WA
- 2014 New Therapeutic Approach to ALS/ Grand Rounds
University of Rochester Medical Center, Rochester, NY
- 2014 Leading multicenter clinical trials/
ANA-TCRC Meeting, Chicago, IL
- 2014 Clinical Trials Methodology Course “Selecting Outcome Measures for Neuroscience
Trials” and “Clinical Trials Networks: NeuroNEXT”
Iowa City, IA (NINDS)
- 2014 ANA Career Development Symposium/ Chair Panel/ Mentor
Baltimore, MD (NINDS)
- 2014 Overview of Challenges for Clinical Trials/ Investigator Research Workshop
Philadelphia, PA (ALSA)
- 2014 2 Care Close Out Meeting
Minneapolis, MN (Huntington Study Group)
- 2015 ALS Therapeutics/ Oh Lectureship/ Grand Rounds
University of Alabama Birmingham, Birmingham, AL
- 2015 ALS Clinical Trials and Why Have They Failed/ Invited Speaker
American Academy of Neurology 67th Annual Meeting, Washington, DC
- 2015 ALS – Update on Therapeutics/NINDS/NIH Clinical Neuroscience Grand Rounds/
Invited Speaker
NIH, Bethesda, MD

- 2015 ALS Update on Therapeutics/ Grand Rounds
Northwestern Medical Hospital, Chicago, IL
- 2015 2015 ALS Therapeutics – Opportunities and Challenges/ Grand Rounds
Johns Hopkins, Baltimore, MD
- 2015 After the ALS ice bucket challenges – hope in therapeutic development/ Grand Rounds/
Invited Speaker
Dartmouth Hitchcock Medical Center, Hanover, NH
- 2015 2015 ALS Therapeutics – Opportunities and Challenges/ Grand Rounds/ Invited
Professorship
Mayo Clinic, Rochester, MN
- 2016 Clinical trials in neurodegenerative disorders: Lessons from ALS and HD/ Invited
Speaker
National Ataxia Foundation, Orlando, FL
- 2016 ALS 2016 – New Therapies and Direction/ Grand Rounds/ Invited Speaker
SUNY Downstate Medical Center, Brooklyn, NY
- 2016 ALS Therapeutics/ Grand Rounds/ Aring Invited Lecturer
University of Cincinnati, Cincinnati, OH
- 2016 Conducting Clinical Trials in an Academic Neurology Setting/ Invited Speaker
8th Annual Translational and Clinical Research Course for Clinician-Scientists
American Neurological Association, Chicago, IL
- 2016 Biomarkers/ Invited Speaker
Clinical Trials Methodology Course
NINDS, Ann Arbor, MI
- 2016 Stem Cell Therapy, RNA and Gene Therapy in ALS/ Invited Speaker
AANEM Annual Meeting
New Orleans, LA
- 2016 Developing Multidisciplinary Clinics for NM Care and Research/ Plenary Speaker
Richard Olney Presentation; AANEM Annual Meeting
New Orleans, LA
- 2016 NINDS/ANA Career Development Symposium/ Invited Speaker
Baltimore, Maryland
- 2017 2nd CNS Clinical Trials Forum/Invited Speaker
Explore Efficient Networks to better position your trial for success. Philadelphia, PA
- 2017 National Institute of Aging/Invited Speaker
ALS – new therapeutics and efficient networks, Baltimore, MD
- 2017 Northeast ALS Consortium/Invited Speaker
Overall Principal Investigator Responsibility, Phoenix, AZ
- 2017 American Academy of Neurology Invited Speaker
Keynote Talk: Keys to success in academic neurology
Research Corner: Grant applications: Working with your Chair and/or grant team from
application to conclusion
- 2017 ALS: New Insights into Biology and Treatments/Grand Rounds Invited Speaker
University of Iowa, Iowa City, IA
- 2017 ALS: New treatments and Insights/Invited Speaker
7th Annual Stanley van den Noort Lecture, University of Irving, Irving, CA
- 2017 American Neurological Association/Invited Speaker
Translational and Clinical Research Course
Conducting Clinical Trials in an Academic Neurology Setting
- 2017 Clinical Trial Methodology Course, NINDS Invited Speaker

- 2017 Biomarkers in Therapy Development Iowa City, IA
Breaking Barriers for Young Women in Science/ Invited Speaker
Society for Neuroscience, Washington, DC
- 2018 ALS Clinical Therapeutic Pipeline/Invited Speaker
Walter Reed Neurology, Washington, DC
- 2018 Merlis Lecture/Invited Speaker
University of Maryland, Baltimore, MD
- 2018 ALS Community Therapy Development Regulatory Pathways Workshop/Invited
Panelist
ALS Association, Washington, DC
- 2018 ALS Therapies: Present Status and the Pipeline/Invited Speaker
8th Annual Les Turner Symposium, Chicago, IL
- 2018 Collaborative Science to Accelerate Therapy Development for ALS/
Invited Speaker
13th Annual Peggy and Gary Edwards Lecture, Houston, TX
- 2018 Amyotrophic Lateral Sclerosis /Invited Speaker
Robert P. Lisak Event, Wayne State University, Detroit, MI
- 2019 Inaugural Neuroscience Symposium/Invited Speaker
Holy Cross Hospital, Fort Lauderdale, FL
- 2019 Presentation of the Sheila Essay Award: An Award for ALS Research /Invited Speaker
2019 AAN Annual Meeting. Philadelphia, PA
- 2019 ALS Finding a Cure: Therapeutics Session Part IV: Brainstorming / Invited Speaker
2019 ALSFAC F2 Meeting, Stamford, CT
- 2019 Robert J. Adams Lecture: ALS Therapeutics 2019 – New Adventures and Hope/Invited
Speaker
Medical University of South Carolina, Charleston, SC
- 2019 Brainstorming Neurodegeneration: Plenary – Accelerating CNS Therapy Development
/Invited Speaker
California Stem Cell Agency, San Francisco, CA
- 2019 Accelerating ALS Therapy Development: The First ALS Platform Trial / Invited Speaker
The ALS Association’s Sixth Annual Hope and Help Symposium, Orlando, FL.
- 2019 Target ALS Annual Meeting: Master Platform Trials & The Healey Center for ALS/Invited
Speaker
Target ALS, Boston, MA
- 2019 Amyotrophic Lateral Sclerosis (ALS) and Related Motor Neuron Diseases/Invited Speaker
Gordon Research Conference, West Dover, VT
- 2019 American Neurological Association. Rat Adams Lectureship
Accelerating ALS Trials: The First Platform Trial in ALS
- 2019 Clinical Trialists Path: Building Teams / Invited Speaker
Society For Neuroscience Meet-the-Clinician-Expert Event, Chicago, IL
- 2019 18th Annual Northeast ALS Consortium Meeting – Platform Trial for the Treatment of
Amyotrophic Lateral Sclerosis (ALS): A perpetual multi-center, multi-regimen clinical
trial evaluating the safety and efficacy of investigational products for the treatment of ALS.
Northeast ALS Consortium, Clearwater Beach, FL
- 2019 9th Annual ALS Clinical Research Learning Institute – The ALS Research Pipeline
Clinical Research Learning Institute, Clearwater Beach, FL
- 2019 144th Annual Meeting of the ANA – Optimizing Clinical Trial
American Neurological Association, St. Louis, MO
- 2020 The Healey ALS Platform Trial - Accelerate Drug Development for ALS Through

- Innovative Trial Designs/ Grand Rounds
University of Pennsylvania, Philadelphia, PA (Virtual)
- 2020 Reimaging ALS Trials – New Science, New Approaches Grand Rounds
Stanford University Medical Center, Stanford, CA (Virtual)
- 2020 ALSFAC Fall Meeting Virtual – Healey ALS Platform Trial
ALS Finding a Cure, Stamford, CT
- 2020 19th Annual Northeast ALS Consortium Virtual Meeting – The Healey Platform Trial
Northeast ALS Consortium, Pasadena, CA
- 2020 19th Annual Northeast ALS Consortium Virtual Meeting – Exploratory Patient-Reported
Outcomes in the Multiple Dose Study of Tofersen (BIIB067) in Participants with ALS
Northeast ALS Consortium, Pasadena, CA
AANEM 2020 Annual Virtual Meeting – ALS Therapeutics: New Treatments and New
Approaches
American Association of Neurologists
- 2020 ANA Professional Development Workshop Virtual Meeting – Negotiations and Contracts
American Neurological Association
- 2020 AANEM 2020 Annual Virtual Meeting – ALS Therapeutics: New Treatments and New
Approaches
American Association of Neuromuscular & Electrodiagnostic Medicine
- 2020 48th Annual Robert B. Aird Professorship Virtual Webinar. New Insights and Approaches
for ALS Therapy Development
Department of Neurology, University of California, San Francisco, CA
- 2020 10th Annual Gilden Lectureship. Virtual Invited Lecture. What’s New in ALS Therapeutics
Dept of Neurology, University of Colorado School of Medicine, Aurora, CO
- 2020 7th International Symposium Virtual Meeting on Focused Ultrasound 2020 – Therapeutic
Advances in Neurodegenerative Disorders: ALS as an example. Invited/Keynote Speaker
Focused Ultrasound Foundation
- 2020 Clinical Research Forum IT Roundtable. Virtual Meeting. Invited Speaker.
Accelerating Drug Development Through Innovative Trial Designs.
- 2020 Excitement and Progress in ALS Therapy Development/Invited Speaker
Takeda Science Virtual Forum
- 2020 ALS Therapy Development 2020/Invited Speaker Wave Science Virtual Forum
- 2020 ALS Therapeutic Updates/Invited Grand Round Speaker, NYU School of Medicine, Dept.
of Neurology, Virtual Forum, New York, NY
- 2021 3rd Annual South Florida Neuroscience, HCH & ALS Patient Symposium
Breaking Update: ALS Clinical Trials
- 2021 Setting up for career success, Virtual NINDS R25/Invited Speaker
Office of Training and Workforce Development NINDS, NIH, Bethesda, MD
- 2021 Virtual Working Group Seminar - Starting New Collaborative Initiatives /Invited Speaker
University of Rochester Medical Center, Monroe County, NY.
- 2021 Michael McDermott Endowed Lectureship in Experimental Therapeutics Virtual /Grand
Rounds/Invited Speaker
University of Rochester Medical Center, Monroe County, NY
- 2021 Ax-S Thursdays Series on Expanding Access to New Medicines – HEALEY ALS Platform
Trials Repurposing Studies, and Clinical Discoveries – accelerating progress and
access/Invited Speaker Ax-S Pharma San Francisco, CA
- 2021 10th Annual Resident Research Day, virtual - New Therapies in ALS/ Invited Speaker
Department of Neurology, University of Miami, Miami, FL

- 2021 Virtual - Motor Neuron Disease/Amyotrophic Lateral Sclerosis - Determinants of clinical response to levosimendan in the REFALS phase 3 study in people with ALS/ Invited Speaker Annual Meeting: World's Premier Neurology Meeting | AAN, Minneapolis, MN
- 2021 2nd Annual George Paulson Lectureship – New Therapies in ALS/Invited Speaker Ohio State University, College of Medicine, Columbus, OH
- 2021 ALS Finding A Cure - Session III: Healey ALS Platform Update, Answer ALS Update. Virtual / Invited Speaker. ALS Finding A Cure, Stamford, CT.
- 2021 Tenth Annual Hope Center Retreat - New Therapies for ALS / Virtual / Invited Speaker Hope Center for Neurological Disorders, St. Louis, MO
- 2021 20th Annual Northeast ALS Consortium Virtual Meeting – NEALS-Affiliated Trial & Project Updates. Northeast ALS Consortium, Virtual
- 2021 The University of Chicago – New Therapeutics for ALS / Virtual / Invited Speaker Neurology Grand Rounds University of Chicago, Chicago, IL
- 2021 Rush University, New Therapeutic Development for ALS / Virtual / Invited Speaker Neurology Grand Rounds Rush University Medical Center, Chicago, IL
- 2021 ALSFAC Virtual Meeting - Healey ALS Platform Trial Update ALS Finding a Cure, Stamford, CT
- 2022 2022 MDA Clinical & Scientific Conference – Relationship of ALS Genes and Single-Nucleotide Polymorphisms with Clinical Outcomes in NurOwn Phase 3 ALS Clinical Trial/Invited Speaker MDA, Nashville, TN
- 2022 Target ALS Annual Meeting: Platform Trials Update/Invited Speaker. Target ALS, Boston, MA
- 2022 The T. Evans Wyckoff Lecture in Science / Invited Speaker The Virginia Mason Medical Center, Seattle, WA
- 2022 Annual Northeast ALS Consortium Meeting – NEALS-Affiliated Trial & Project Updates. Clearwater, FL (In-Person)
- 2022 Yale School of Medicine. Neuroscience Grand Rounds / Invited Speaker / In-Person Yale University, New Haven, CT
- 2023 13th Annual California ALS Research Summit. The Healey Platform Trial Innovative Trials and Other Approaches/Invited Speaker ALS Golden West Chapter Association, San Francisco, CA.
- 2023 NIH StrokeNet Grand Rounds. Cincinnati Trial Platforms; EVT Platform & NeuroNEXT Platform Trials/ Virtual/Invited Speaker. University of Cincinnati, Cincinnati, OH.
- 2023 St. Louis University Grand Rounds. New Therapies in ALS. Virtual/Invited Speaker. Washington University of St. Louis, St. Louis, MO
- 2023 ASENT 2023 Annual Meeting. ALS Outcomes and Clinical Trial Design. Virtual/Invited Speaker. American Society for Experimental Neurotherapeutics, Haddonfield, NJ
- 2023 NEALS Clinical Trials Workshop. ALS Clinical Trials of the Future. Invited Speaker / In-Person. Northeast Amyotrophic Lateral Sclerosis Consortium | NEALS, Boston, MA
- 2023 2023 Muscular Dystrophy Association Clinical & Scientific Conference. Invited Speaker / In-Person. Muscular Dystrophy Association. Dallas, TX.
- 2023 Annual Northeast ALS Consortium Meeting – NEALS-Himalaya trial: Double-blind, placebo-controlled trial of RIPK1 inhibitor (SAR443820) in ALS. Clearwater, FL (In-Person).

International

- 1995 Update on familial ALS/ Invited Speaker American Congress of Neurology, Guatemala City, Guatemala

- 2001 Clinical trial of Minocycline in Huntington's Disease/ Invited Speaker
Huntington's Disease Study Group Annual Meeting, Toronto, Canada
- 2002 From bench to bedside and back: Therapies in ALS/ Invited Plenary Speaker
International Motor Neuron Disease Symposium, Melbourne, Australia
- 2002 Experimental therapies in ALS/ Invited Expert Panelist
Australia Motor Neuron Disease Society, Melbourne, Australia
- 2002 Clinical trial of Topiramate in ALS/ Invited Speaker (abstract)
International Motor Neuron Disease Symposium, Melbourne, Australia
- 2003 Dementia and ALS/Invited Speaker Expert Panel
Novartis Neuroscience Advisory Board Meeting, Seville, Spain
- 2004 Three invited presentations
Celebrex in ALS (abstract)
Ask the Experts Clinical Trials in ALS
Clinical Symposium highlights
International Motor Neuron Disease Symposium, Philadelphia
- 2006 ALS trials challenges and the future/ Invited Speaker
2nd Annula Symposium de la Fondation Andres-Delambre on ALS, Québec, Canada
- 2006 Phase IIA clinical trial of Arimoclomol in ALS (abstract)
International Motor Neuron Disease Symposium, Yokohama, Japan
- 2007 ALS lessons from past trials/ Invited Speaker
3rd Annual Symposium de la Fondation Andres-Delambre on ALS, Montréal, Canada
- 2007 Longitudinal metabolomic profiling in plasma from patients with ALS (abstract)
International Motor Neuron Disease Symposium, Toronto, Canada
- 2008 Advances in ALS Therapy, A Brave New World/ Invited Speaker
- 2009 4th Annual Symposium de la Fondation Andres-Delambre on ALS, Montréal, Canada
Two Presentations
Ceftriaxone in ALS: results of stages 1 and 2 (abstract)
KNS760704-CL-201, Part 1: a 12 week phase 2 study of the safety, tolerability and clinical effect in ALS subjects (abstract)
20th International Motor Neuron Symposium, Berlin Germany
- 2010 AAN Clinical trial research fellowship: Case study of success/ Two Presentations/
Invited Speaker
AAN/NINDS Research Career Development Symposium
A Phase 2 study of KNS760704 in ALS/ Invited Plenary Speaker
American Academy of Neurology Annual Meeting, Toronto, Canada
- 2010 More power to you: Impact of trial design on adherence and outcome/ Invited Plenary
Speaker
21st International Motor Neuron Symposium, Orlando
- 2010 Biobanks and Standard Operating Procedures for Biomarkers in ALS/ Invited Speaker
International Symposium on Biomarkers in ALS Ulm, Germany
- 2010 Present and future of ALS clinical research/ Invited Plenary Speaker
1st ARISLA Conference, Milan, Italy
- 2011 Update on ALS Therapies/ Invited Speaker
Suna Kirac Conferences on Neurodegeneration, Istanbul, Turkey
- 2012 ALS: Exploratory and Confirmatory Trials/ Invited Speaker
Symposium sur la SLA de la Fondation Andre-Delambre
Québec
- 2013 Novel Approaches for the Treatment of Amyotrophic Lateral Sclerosis/ Invited Speaker
The International Conference on Alzheimer's & Parkinson's Diseases, Florence, Italy

- 2013 Academic Career and Family: Can you have it all?/
Mexican Academy of Neurology. Merida, Yucatán, Mexico
- 2013 Neuromuscular Diseases/NEALS
Mexican Academy of Neurology. Merida, Yucatán, Mexico
- 2014 Infrastructure of the trial: ALS registry in the US Northeast/ Invited Speaker
MAN/ANA Symposium. Cancun, Mexico
- 2016 ALS Trials and Trialists: New Opportunities and Approaches to Find Treatments for Our
Patients./ Invited Speaker
ENCALS Meeting 2016. Milan, Italy
- 2016 Challenges and Opportunities in ALS Clinical Trials/ Invited Speaker
4th Annual CTNI Europe Symposium. Stresa, Italy
- 2016 ALS SOD1 trials/ Invited Speaker
The Lancet Neurology Conference. London, United Kingdom
- 2016 Mexican Academy of Neurology Conference/ Invited Speaker
Pan American Congress of Neurology. Cancún, Mexico
- 2016 Neurofilaments in ALS clinics and beyond/ Invited Speaker
27th International Symposium on ALS/MND. Dublin, Ireland
- 2017 ALS - targeted treatments based on underlying biology. New science and new hope
Finland Centennial: Building the future together/Invited Plenary Speaker,
Academic Medicine and Pharma Medical Center Helsinki
- 2017 The crisis we want: Are healthcare systems prepared for a disease modifying drug?/
Panel Moderator
World Neuroscience Innovation Forum, London, UK
- 2017 ALS therapeutics and opportunities for collaboration. Invited Speaker
ALS Clinical and Basic Research in China and the United States Beijing, China
- 2018 Amyotrophic Lateral Sclerosis – Alagoas in Motion / Invited Speaker
First International Symposium. Maceió, Brazil
- 2019 Florey Institute of Neuroscience and Mental Health/ Invited Speaker
Fight MND. International Motor Neuron Disease Symposium. Melbourne, Australia
- 2019 Macquarie University and the Faculty of Medicine and Health Sciences/Invited Speaker
International Symposium. Sydney, Australia
- 2019 Accelerating ALS-treatment discovery: targets, therapies and platform trials. /Invited
Speaker. International Conference on Neurological Disorders and Neurorestoration.
Dubrovnik, Croatia
- 2019 Accelerating Therapy Development: Lesson From NeuroNEXT Phase II Network and
ALS/ Invited Speaker
14th International Conference in Alzheimer's & Parkinson's Diseases. Lisbon, Portugal
- 2019 Master Platform Trial: Invited Speaker. Symposium sur la SLA de la Fondation Andre-
Delambre, Montréal, Canada
- 2019 New Therapeutic Approaches to ALS. /Invited Speaker. VII Annual Symposium.
Rome, Italy
- 2020 ALS Platform Trial Initiative: Accelerating Therapy Development. /Invited Speaker
AAT-AD/PD 2020. Virtual Lecture, Vienna, Austria
- 2020 ALS Therapies – Therapies in Late Stage Testing Therapeutic Pipeline Healey ALS
Platform Trial / Virtual Grand Rounds
University of Alberta, Edmonton, Alberta, Canada
- 2020 The Refals Phase 3 Study of Levosimendan in Patients with ALS
31st International Symposium on ALS/MND/Virtual Lecture
- 2020 NurOwn ALS Phase 3 Clinical Trial Update

- 2020 31st International Symposium on ALS/MND/Virtual Lecture
Healey ALS Platform Trial Canadian ALS Network/Virtual Lecture
- 2021 III International Symposium on Amyotrophic Lateral Sclerosis/Virtual/Invited Speaker
International Symposium, Brazil
- 2021 III Congreso SOLANE, Latin American Society for Neuromuscular Diseases –
Virtual/Invited Speaker
International Symposium, Chile
- 2021 PACTALS Nagoya Symposium, Hot Topics of Clinical Trials for ALS / Virtual / Invited
Keynote Speaker
Aichi Medical University, Yazakokarimata, Nagakute, Aichi, Japan
- 2021 PACTALS 2021 Nagoya Symposium, Current Progress of Therapeutic Development for
ALS / Virtual / Invited Speaker
Aichi Medical University, Yazakokarimata, Nagakute, Aichi, Japan
- 2022 France Biotech Conference, “ALS & Motor Neuron Diseases: What Avenues for the
Future?”/Invited Speaker France Biotech (Virtual)
- 2022 Neuromuscular Day Event. Therapies for ALS/Invited Speaker
University of Toronto, Toronto, Canada (Virtual)
- 2022 7th annual Russian conference for patients with ALS. ALS Trials in Development for
Russian ALS Foundation/Invited Speaker. Russian Charity ALS Foundation, Moscow,
Russia
- 2022 2nd International Conference on Neurological Disorders and Neurorestoration. Progress
in ALS Therapeutics /Invited Keynote Speaker. Dubrovnik, Croatia.
- 2022 The HEALEY ALS Platform Trial - Accelerating Innovation for a Cure. Invited
Speaker.
International Alliance of ALS/MND Associations. (Virtual)
- 2022 44th Annual Convention of the Philippine Neurological Association (PNA). State of the
Art and Future Trends in Motor Neuron Disease. Invited Speaker. Virtual.
Philippines Neurological Association, Philippines.
- 2023 26th World Congress of Neurology (WCN). Clinical Trial Progress in ALS. Invited
Speaker. In-person. Montreal, Canada.
- 2023 ALS/MND Science Day. Update on Clinical Trials/Therapeutics. Keynote Speaker. In-
Person. German Network of ALS.MND, Frankfurt, Germany.
- 2023 ALS/MND Symposium. C 62Two-year results from the open label extension of VALOR:
Tofersen in adults with SOD1 ALS. Invited Speaker. In-Person. (MNDA) Motor Neuron
Disease Association, Basel, Switzerland.
- 2023 Cytokinetics Symposium at ALSMND. Invited Speaker. In-Person. (MNDA)
Cytokinetics, Inc, San Francisco, California.

Report of Clinical Activities and Innovations

Current Licensure and Certification

1995 Massachusetts Medical License

Practice Activities

1996-2004	Ambulatory Care	Huntington's Disease Clinic, MGH	Two sessions a month
1996-	Ambulatory Care	ALS Clinic, MGH	One session a week
1998- 2009	Inpatient attending	Neurology Service, MGH	One month a year
2007-11	Inpatient attending	Neurology Service, MGH	Two weeks a year

Clinical Innovations

New England Telemedicine Care Program: ALS telemedicine collaborative started in late 2010 to improve access to care and clinical research for people with amyotrophic lateral sclerosis living in New England.

- ALS ONE program: Hospitals in MA coordinate care of patients with ALS
- ALS House call program
- ALS parenting at a challenging time program
- Expanded Access Program in ALS

Report of Technological and Other Scientific Innovations

- Biomarker assay for ALS US patent 11/711,518, filed 2/27/07; and International Patent Application No. PCT/US2007/004998 filed on 2/27/07. Cudkowicz ME, Brown RH and staff from Metabolon, inventors. Biomarkers for Amyotrophic lateral Sclerosis and Methods Using the Same

The present invention provides various biomarkers of amyotrophic lateral sclerosis (ALS) as well as providing various methods of using the biomarkers. Methods for biomarker usage include diagnosis, determining predisposition, monitoring progression/regression, assessing efficacy of compositions for treating ALS, screening compositions for activity in modulating biomarkers of ALS, treating ALS, as well as other methods based on biomarkers of ALS.
- ATLIS – an apparatus to quantitatively measure strength US patent 11-715200, filed 3/7/07. Andres P, Cudkowicz ME, MGH inventors. Isometric Strength Testing Apparatus.

Accurate Test of Limb Isometric Strength (ATLIS) was designed to be portable, quick, and easy to use device measuring isometric strength. This device could more precisely measure whether an experimental drug slows the progression of ALS or other neuromuscular diseases, allowing more drugs to be tested quicker and at less expense.
- Role of Microglia in Retinitis Pigmentosa NIH R01EY027921 (PI: Weiner HL, /Co-PI: Butovsky O, Cudkowicz ME.

The focus of this grant proposal is to characterize retinal microglia and how they regulate and/or participate in retinal damage in both animal models of retinitis pigmentosa and in eyes from human subjects. We will investigate whether retinitis pigmentosa in animal models can be treated by specifically targeting and modulating microglia. We will use new technology and approaches to understand features of microglia cells that can then be exploited to develop novel microglia-targeting therapies to treat humans with retinitis pigmentosa.

Randomized,
Double-Blind,
Placebo-
Controlled Trial
of Arimocloamol
in Rapidly
Progressive SOD1
ALS

MGH Invention 25521 (PI: Cudkowicz, ME, Co-PI Atassi N, Schoenfeld D.
The clinical trial was funded by the FDA and was joint initiative with Dr Michael
Benatar. Dr. Cudkowicz led the first trials of Arimocloamol in ALS. This was a
study just in people with rapidly progressive SOD1 ALS.

Report of Education of Patients and Service to the Community

Activities

- 1999 Therapeutic strategies in ALS/ Invited Speaker
New York ALS Association Symposium, New York City
- 1999 Therapeutic advances in ALS/ Invited Speaker
Rhode Island ALS Association Symposium, Providence
- 2001 ALS clinical research update/ Invited Speaker
ALS Hope Foundation, Philadelphia
- 2003 Updates in the new millennium/ Invited Speaker
ALS Association of New Hampshire, Portsmouth
- 2003 Therapeutic advances in ALS/Invited Speaker
Muscular Dystrophy Association of Rhode Island, Pawtucket
- 2005 Therapies for ALS Update/ Invited Speaker
ALS Association New York Chapter, New York City
- 2006 ALS Updates: Genes, mechanisms and therapies/ Invited Speaker
Rhode Island ALS Association Symposium, Providence
- 2010 ALS Treatments and Hopes: Roadmap to the Cure/ Invited Speaker
ALS Association May Advocacy Day, Washington
- 2010 ALS Therapeutics, a time for hope/ Invited Speaker
ALS Therapy Development Foundation Research Day, Cambridge
- 2010 Therapeutics in 2010/ Invited Speaker
ALS Association Ask the Experts, Orlando
- 2011 Multidisciplinary Care and Therapeutic Advances for People with ALS/ Invited
Speaker
Scholarship in Medicine Distinguished Speaker Series, Maine General Medical
Center, Waterville
- 2011 Annual Leadership Summit/ Invited Speaker
ALS Therapy Development Institute, Cambridge
- 2013 The Northeast ALS Consortium
ALS Clinical Trials: Pipeline Update Summer 2013
- 2014 MGH ALS Program: drugs, genes, stem cells: therapeutic revolution
- 2014 The Northeast ALS Consortium
Drugs, gene therapy, stem cells: Therapeutic revolution in ALS
- 2015 The Northeast ALS Consortium
The ALS Research Pipeline
- 2016 ALS Association National: ALS Advocacy Day & Public Policy Conference/ Invited
Speaker
Current trials, stem cell trials, Retigabine and ALS ACT

- 2017 Status of ALS therapies/Invited Speaker
Florida ALS Association patient event
- 2018 ALS Therapy Update/Invited Speaker
Bonita Spring, FL patient event
- 2020 The McCourt Foundation Education Update/Invited Speaker
Innovations for a Cure: Clinical & Research Advances in ALS
- 2020 ALS Roundtable tele series/Invited Speaker
What's New in ALS Therapy Development
ALS Association Greater Chicago Chapter, Chicago, IL (Virtual)
- 2020 Argentina video-conference for ALS Community/Invited Speaker
ALS Therapies
Asociacion Ela Argentina, Buenos Aires, Argentina
- 2020 Webinar on Innovation in Trial Design: Bringing together a community to innovate the
ALS clinical trial landscape. International MDN Alliance
- 2020 NEALS Patient Webinar on the REFALS Phase 3 Study Levosimendan in ALS.
- 2020 10th Annual ALS Clinical Research Learning Institute Virtual Meeting – The ALS
Research Pipeline. Clinical Research Learning Institute (Virtual)
- 2021 ALS Drug Development Webinar Series Part 6: Patient Engagement in Clinical Trials
Meeting (Virtual)
- 2021 ALS Your Day 7th Annual Care & Research Symposium – Platform Trials and
Expanded Access Programs.
- 2021 Everything ALS Webinar Series: New Therapies for ALS Meeting. (Virtual)
- 2022 Inaugural I am ALS Action Summit. Today's Experience Finding and Getting into
Clinical Trials/Invited Panelist. (Virtual)
- 2022 ALS TDI Town Hall: Supplement Use for People with ALS Meeting ALS Therapy
Development Institute, Watertown, MA (Virtual)
- 2022 Denali Therapeutics ALS Forum: The State of ALS Therapeutics Today /Invited
Panelist. (Virtual)
- 2022 ALS TDI Summit – New Innovations in ALS Therapeutics Meeting. Invited Speaker
ALS Therapy Development Institute. Watertown, MA (Virtual)
- 2022 Annual ALS Clinical Research Learning Institute Meeting – The ALS Research
Pipeline. Clearwater, FL. (In-person).
- 2023 Annual ALS Clinical Research Learning Institute Meeting – The ALS Research
Pipeline. Clearwater, FL. (In-person).

Educational Material for Patients and the Lay Community

Books, monographs, articles and presentations in other media

- | | | |
|------|-----------------------------------------------------------------------------------------------------|----------------------------------------------------------------------------------------------------------------------------------------------------|
| 2008 | ALS diagnosis, clinical care and clinical
research video | Lecturer for a videotaped series
utilized as a training tool to establish
ALS centers in China |
| 2010 | Clinical Trials 101 and Ceftriaxone
Clinical Trials | Lecturer in a webinar for patients with
ALS |
| 2011 | The effects of dexpramipexole (KNS-
760704) in individuals with amyotrophic
lateral sclerosis | Speaker for a Nature Medicine,
podcast
http://www.nature.com/nm/podcast/index.html |

Educational material or curricula developed for non-professional students

2011	ALS Trial Update	Lecturer in a webinar, ALS Therapy Development Institute
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Recognition

2007	Medical All Star Awardee	Boston Red Sox
2007, 2013-2016 2019, 2020 2022	Top Doctors	Boston Magazine
2021	Castle Connolly Exceptional Women in Medicine	New York Magazine
2022	Exception Women in Medicine	The American Registry
2023	Top Doctors	The American Registry
2023	Exceptional Women in Medicine	The American Registry

Report of Scholarship

Peer reviewed publications in print or other media

Research Investigations

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14. Marder K, Ahao H, Myers RH, **Cudkowitz ME**, Kayson E, Kieburtz K, Orme C, Paulsen J, Penney J, Siemers E, Shoulson I, and the Huntington Study Group. Rate of Functional decline in Huntington's Disease. *Neurology*. 2000; 54(2):452-458.
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Narrative Report

My research and clinical activities are dedicated to the study and treatment of patients with neurodegenerative disorders, My research focus has been on developing novel treatments for Amyotrophic lateral sclerosis (ALS) and Huntington's Disease (HD). I designed and initiated several human clinical trials to evaluate therapies that have shown promise in preclinical studies. I built a neurology clinical trial unit to conduct high quality, informative and efficient clinical research studies. Helping young investigators become leaders in clinical research, working closely with lab based scientists to develop treatments and finding ways to accelerate the testing of high impact therapies in people with neurological disorders are some of my passions. I have led and am leading multisite networks to collaboratively develop new therapies and biomarkers for neurological disorders. As a co-founder and prior chair, the Northeast ALS Consortium (NEALS), we have developed outcome measures, trained over 100 site staff and investigators, and tested several novel therapies in ALS. Several recent studies to date led have yielded positive phase II results (nuedexta, CK-2017357, NurOwn and mexiletine).

My research efforts also include studies risk factors for the development of ALS and biomarkers. I teach neurology residents and mentor neuromuscular and ALS research fellows. I currently have a U01 from the National Institute of Neurological Disorders and Stroke (NINDS) as the Coordination Center for NeuroNEXT. Within this network we are currently coordinating 8 national trials. NeuroNEXT is a

phase 2 network designed to be efficient and enable the best ideas for neurotherapeutics to be tested in people. Through this network we trained and mentored dozens of investigators in phase 2 trial design and several new investigators on how to lead and conduct multicenter trials. We also set up a central IRB for the network and a master contract system that greatly reduced start up time and administrative burden. Every trial involves patient advocates, and each trial has enrolled ahead or on schedule. Data on natural history and outcome measures from the first completed study was instrumental to the FDA for their review and approval of the first drug for spinal muscular atrophy. Taken together, my research efforts constitute a comprehensive program to translate basic science breakthroughs into effective treatments for patients with neurological disorders.

The NEALS consortium, which I co-direct for 18 years was formed in 1995, consists of over 100 clinical sites in the United States and Canada dedicated to performing joint academic led clinical trials in ALS. All data management and clinical trial coordination for the group is performed under my direction at MGH. In conjunction with the NEALS consortium, I planned and completed over 20 multi-center clinical trials and biomarker initiatives in ALS. I am currently mentoring and on the steering committee for several new trials in ALS including phase II trials of retigabine, actemra, and the combination of tudca and sodium phenylbutrate. and a phase I study of an antisense oligonucleotide to superoxide dismutase (SOD1) in people with familial ALS. Within NEALS, we provide training and guidance to member clinical trial sites. We trained over 50 new principal investigators on how to design, lead and conduct multicenter trials. De-identified data from NEALS studies are shared in an open-source system called ProACT, which won an innovation award and has led to several publications and new insights into the disease. Similarly, all biosamples collected in our studies are shared openly. The biorepository is housed and managed at MGH and the Barrows Neurological Institute.

In 1994, along with Dr. Greenberg, I established the Neurology Clinical Research Institute (NCRI) at MGH. This institute that has approximately 95 full time staff who coordinate and perform data management for trials in several neurological disorders. I mentor and assist others clinical investigators in the Department of Neurology and throughout the United States with study trial design, funding applications, and regulatory compliance.

There is a critical need in neurology for highly trained clinical investigators who work collaboratively with basic scientists to develop new therapeutic strategies in patients. One of my passions to mentor young neurologists in clinical investigation. In collaboration with Drs. Ravina and Piantadosi, I was awarded a contract from the NINDS to create an annual one-week training course in Clinical Trial Methods for fellows and junior faculty members in Neurology (www.neurologytrials.org). This course was very successful and is now complete. I am an active participant in a similar NINDS sponsored course now led by Dr. William Meurer from the University of Michigan. I am actively involved in mentoring and teaching students at many levels, including high school students, college graduates, medical students, neurology residents, clinical fellows and junior faculty members.