

TIMOTHY M. MILLER, MD, PHD

DATE: October 1, 2025

PERSONAL INFORMATION:

DOB, 5/30/1969

CITIZENSHIP: USA

CONTACT INFORMATION:

Neuromuscular Division

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PRESENT POSITION: Professor of Neurology, Washington University in St. Louis
Vice Chair of Research in Neurology, Washington University in St. Louis
Co-Director, ALS Center, Washington University in St. Louis

EDUCATION:

1987-1991	University of Virginia	Chemistry/French	B.S.
1991-1998	Washington University	Medicine/Neuroscience	MD/PhD

ACADEMIC POSITIONS:

1998-1999	Internal Medicine Internship, University of California, San Francisco
1999-2002	Neurology Residency, University of California, San Francisco
2002-2003	Neuromuscular Fellowship, University of California, San Francisco
2003-2007	Clinical Scientist Training Program, UCSD Post-doctoral research, Laboratory of Don Cleveland
2007-2013	Assistant Professor of Neurology, Washington University School of Medicine
2014-2016	Associate Professor of Neurology, Washington University School of Medicine
2017-current	Professor of Neurology, Washington University School of Medicine
2021-current	Vice Chair of Research in Neurology, Washington University School of Medicine

UNIVERSITY AND HOSPITAL APPOINTMENTS AND COMMITTEES:

University of California San Diego, Attending Privileges, 2003-2007

Barnes-Jewish Hospital, Attending Privileges, 2007-current

Neuroscience Admissions Committee, 2007-2012

Medical Scientist Training Program Admissions Committee, 2017-2021

Committee on Research Integrity, 2019 – 2023

Hope Center for Neurological Disorders – Faculty

Muscular Dystrophy Association Clinic – Physician

Co-Director, ALS Center at Washington University in St. Louis

Graduate Program in Neurosciences – Faculty
Graduate Program in Cell Biology - Faculty
Graduate Program in Biochemistry – Faculty
Research Mentorship Committee - Faculty

LICENSES AND CERTIFICATIONS:

1999-2007 Medical License, State of California
2004 Certified in Psychiatry and Neurology, American Board of Psychiatry and Neurology
2005 Certified in Neurophysiology, American Board of Psychiatry and Neurology
2007 Medical License, State of Missouri
2012 Certified in Neuromuscular Medicine, American Board of Psychiatry and Neurology

HONORS AND AWARDS:

1987 Westlake Scholarship, University of Virginia
1987 Echols Scholar, University of Virginia
1991 Alpha Chi Sigma Chemistry Award, University of Virginia
1991 Raven Fellowship for research at the CNRS, Gif-Sur-Yvette, France
1991 B.S. Chemistry with High Distinction, University of Virginia
1998 Medical Scientist Training Program, Washington University
1998 Needleman Award for Pharmacology Research, Washington University
2002 Chief Resident in Neurology, University of California, San Francisco
2003 Golseth Young Investigator Award, AANEM
2007 Director of the Christopher Wells Hobler Laboratory for ALS Research
2011 Elected Member of ANA
2013 Lisa Krivickas Lecturer, Massachusetts General Hospital Neurology
2014 Toni Diamond Award for ALS Research, Muscular Dystrophy Wings Over Wallstreet
2015 David Clayson Professorship
2016 Elected Member of American Society of Clinical Investigation
2018 Sheila Essey Award for ALS Research, American Academy of Neurology
2018 Spirit of Hope Award, Hope Center, Washington University
2018 Richard K. Olney Invited Lecture, American Academy of Neuromuscular and Electrodiagnostic Medicine Annual Meeting
2019 Healey Center International Prize for Innovation in ALS, MNDA International Symposium on ALS/MND Perth, Australia
2022 Target ALS Rebecca Luker Courage Award
2023 Rainwater Prize for Outstanding Innovation in Neurodegenerative Disease Research, Rainwater Charitable Foundation, Lille, France
2023 Elected to the National Academy of Medicine (NAM)

SERVICE AND PROFESSIONAL MEMBERSHIPS:

American Academy of Neurology
Society for Neuroscience
American Neurological Association
American Neurological Association, Scientific Program Advisory Committee
American Society of Clinical Investigation
Scientific Advisory Board Co-Chair, Northeast ALS Consortium
Co-Chair, Northeast ALS Consortium (2018-2022)
Science Advisory Council for the ALS Center at Massachusetts General Hospital
Scientific Advisory Board for the Robert Packard Center for ALS Research
Therapy Evaluation Committee Chair for Healey ALS Platform Trial
National Academy of Medicine

SERVICE TO PROFESSIONAL PUBLICATIONS:

Editorial Board, Experimental Neurology, 2012-present
Reviewer for Neuron, Journal of Experimental Medicine, Journal of Clinical Investigation, Cell Reports, Nature Medicine, Journal of Neuroscience, Experimental Neurology, Annals of Neurology, Neurology, JAMA, PNAS, Science Translational Medicine
AAN Annual Meeting abstract reviewer, 2009-2013

GRANT REVIEWS:

NIH NCATS, Ad Hoc reviewer
NIH CNBT, Ad Hoc reviewer, 2016-present
NIH DNDA, R21 grant reviewer, 2018
Muscular Dystrophy Association Ad Hoc reviewer, 2010-present
Muscular Dystrophy Association Review Committee, 2017-present
ALS Association, fellowship review, 2013, 2015, 2016
Hope Center for Neurological Disorders
Alzheimer's Disease Research Center, Washington University
ALS Association, Canada
Weston Brain Foundation

INVITED LECTURES AND PRESENTATIONS:

2008 Sporadic Neurodegeneration Symposium, Boston, Massachusetts
2008 Translational Neuroscience Symposium, University of Chicago, Chicago, Illinois
2009 Neurodegenerative Disease Keystone Meeting, Keystone, Colorado
2009 Muscle Study Group Meeting, New York, New York
2010 NIH/FDA Conference on Antisense Oligonucleotide Therapies in Neuromuscular Disease, Washington D.C.
2010 Neurodegenerative Disease Symposium, Paris, France
2010 HD Therapeutics Conference: A Forum for Drug Discovery and Development (CHDI), Palm Springs, California
2010 Andre Delambre Symposium, Quebec, Canada
2011 Kansas University Neurology Department Grand Rounds
2011 Neurodegenerative Disease Symposium, University of Massachusetts
2011 Neurotherapeutics Institute Symposium
2011 Instituto Gulbenkian de Ciencia, Course Lecturer and Seminar Speaker, Lisbon, Portugal
2011 International ALS Conference: To Promote Clinical Research to Find the Pathogenesis and Cause of ALS, Tarrytown House, New York, New York
2012 ALS Association Drug Discovery Meeting, Banbury Conference Center, Cold Spring Harbor, New York
2012 ALS Society of Canada Annual Meeting, Plenary lecture
2012 Families of SMA Research Conference, Bloomington, Minnesota
2012 Neurodegeneration Meeting, Cold Spring Harbor, New York
2013 Lisa Krivickas ALS Lecture (Massachusetts General Hospital), Boston, Massachusetts
2013 Plenary Lecture, ALS-MND Meeting, Milan, Italy
2014 University of Florida, Genetics Seminar
2014 Japanese Neurologic Society, Japan
2014 Andre Delambre ALS Meeting, Montreal, Canada
2014 Gordon Conference: Mechanisms of Epilepsy, West Dover, Vermont
2014 Neurodegeneration Meeting, Cold Spring Harbor, New York
2014 Alzheimers Association International Conference, Copenhagen, Denmark

2014 International FTD Meeting, Vancouver, Canada
2015 AAN Hot Topics Plenary
2015 Tau Consortium Investigators Meeting, San Francisco, California
2015 Annual NEALS Meeting, Clearwater Beach, Florida
2016 Annual Robert Packard Center for ALS Research Meeting, Baltimore, Maryland
2016 Tau Consortium Investigators Meeting, Fort Worth, Texas
2016 Annual NEALS Meeting, Clearwater Beach, Florida
2016 ALS Association Annual Educational and Scientific Symposium, Chicago, Illinois
2016 Packard Center Annual Meeting – Baltimore, Maryland
2016 Target ALS – New York, New York
2016 MDA ALS Symposium
2016 Northwestern Neurology Research Seminar Series, Chicago, Illinois
2016 Tau Consortium Investigators Meeting, Denver, Colorado
2016 AANEM Annual Meeting, New Orleans, Louisiana
2016 NEALS Meeting, Clearwater Beach, Florida
2016 ALSA Drug Development Workshop, Clearwater, Florida
2016 ALSA Chicago Symposium, Chicago, Illinois
2016 SFN Meeting, San Diego, California
2016 Neurodegeneration Meeting, Cold Spring Harbor, New York
2016 ALS MND Meeting
2017 Tau Consortium Investigators Meeting, Las Colinas, Texas
2017 Packard Presentations, Baltimore, Maryland
2017 ALS Finding a Cure, Connecticut
2017 Mayo Clinic Visiting Faculty
2017 Tau Consortium Investigators Meeting, Denver, Colorado
2017 NEALS Annual Meeting, Clearwater Beach, Florida
2017 ANA Meeting, San Diego, California
2017 UCSF Neuroscience Lecture, San Francisco, California
2017 ALSA Meeting, Boston, Massachusetts
2018 Tau Consortium Meeting, Irving, Texas
2018 NINDS Neurology Grand Rounds, Bethesda, Maryland
2018 Packard Center Annual Symposium
2018 Grand Rounds at Johns Hopkins University, Baltimore, Maryland
2018 Award Presentation, AAN Meeting, Los Angeles, California
2018 ALSA Talk, New York, New York
2018 ICNMD, Vienna, Austria
2018 Tau Consortium Investigators Meeting, Denver, Colorado
2019 Tau Consortium Meeting, Irving, Texas
2019 TDP-43 Biomarkers for FTD and ALS Workshop, South Beach Miami, Florida
2019 NYU Neurology Grand Rounds Presentation, New York, New York
2019 UCLA Integrative Center for Neurogenetics Seminar, Los Angeles, California
2019 AAN Annual Meeting, Data Blitz Presentation, Philadelphia, Pennsylvania
2019 ENCALS Meeting, Tours, France
2019 Annual UC Irvine Health Neuromuscular Colloquium, Huntington Beach, California
2019 DIAD Family Conference, Los Angeles, California
2019 Tau Consortium Investigators Meeting, West Dover, Vermont
2019 Muscle Study Group Meeting, Salt Lake City, Utah
2019 NEALS Annual Meeting, Clearwater Beach, Florida
2019 MNDA International Symposium on ALS/MND, Perth, Australia
2020 Neurology Grand Rounds, Emory University School of Medicine, Atlanta, Georgia
2020 Neuroscience GIDP Seminar Series, University of Arizona

2020 Tau Consortium Meeting, Washington D.C.
2020 20th Annual Packard Center ALS Research Symposium, Baltimore, Maryland
2020 NEALS Webinars: Simplifying the Search - How to Find the Right ALS Clinical Trial for You; Impact of COVID-19 on ALS Clinical Research; NEALS COVID-19 Webinar for Patients; Conducting ALS Clinical Trials During the COVID-19 Pandemic
2020 Advisory Council Spring Meeting, Healey Center, Boston, Massachusetts
2020 Virtual Talk, ALS Association of Argentina, International Alliance of ALS/MND Associations
2020 Virtual Talk, Neurology Symposium, Leuven, Belgium
2020 Virtual Talk, National Institute on Aging Workshop
2020 Virtual Presentation, Genentech Summit, San Francisco, California
2021 Virtual Presentation, Icahn School of Medicine at Mount Sinai
2021 Virtual Neurology Grand Rounds, University of Pittsburgh, School of Medicine
2021 Virtual Meeting, Tau Consortium
2021 Virtual Neurology Grand Rounds, Yale University
2021 Virtual Presentation and Meeting, The 21st Annual Packard Center Symposium
2021 Virtual Presentation, AAN 2021 Annual Meeting
2021 Virtual Presentation, ICNMD 2021
2021 Virtual Presentation, Midwest Motoneuron Consortium
2021 Virtual Presentation, PACTALS 2021, Nagoya, Japan
2021 Virtual Presentation, Science Friday Talk, Kansas City University
2021 Virtual Presentation, NEALS Annual Meeting
2022 Virtual Presentation, ANA 2021 Annual Meeting
2022 Grand Rounds Guest Lecturer, University of Chicago, Chicago, Illinois
2022 22nd Annual Packard Center Symposium, Remote Presentation, Baltimore, Maryland
2022 ENCALS (European Network to Cure ALS) Annual Meeting, Edinburgh, Scotland
2022 Denali Therapeutics ALS Forum, Guest Speaker, San Francisco, California
2022 World Federation of Neurology, ALS/MND Satellite Virtual Symposium
2023 ALS Research Summit, San Francisco, California
2023 30th Adler Symposium, San Diego, California
2023 23rd Annual Packard Center, Baltimore, Maryland
2023 ALS Canada Research Forum, Toronto, Canada
2023 EuroTau, Lille, France
2024 Packard Center ALS Research Symposium, Baltimore, Maryland
2024 Virtual Presentation, NIH Gene Therapy Special Interest Group
2024 Virtual Presentation, Howard University Department of Physiology Seminar
2024 Virtual Presentation, Boston University Grand Rounds
2024 UT Southwestern, OBI Grand Rounds, Dallas, Texas
2024 AAIC Plenary Invitation, Philadelphia, Pennsylvania
2024 ANA Annual Meeting, Opening Plenary speaker, Orland, Florida
2024 Virtual Presentation, 2024 Annual NEALS Meeting
2024 Tetra-Institutional Alzheimer Disease Research Seminar Presenter, New York, NY
2024 Andre Delambre Symposium Presenter, Montreal Canada
2025 Duke Grand Rounds, Speaker, Durham, NC
2025 Trefethen Lectureship at UCSF, San Francisco, California
2025 2025 Training in Neurotherapeutics Course at UC Davis, Lecturer, Sacramento, California
2025 Neurology Podcast, Invited interviewee
2025 BioMarin Retreat, Keynote Speaker, Monterey, California
2025 ENCALS Annual Meeting Turin, Italy Presenter
2025 ALS Nexus Conference, Dallas, Texas Speaker
2025 TCIM San Diego, California Presenter
2025 Teleconference ALS Association Argentina

REGIONAL AND OTHER INVITED PRESENTATIONS:

- 2007 6th Leonard Berg Symposium: Novel Therapies for Protein Misfolding Disorders
- 2008 Washington University Neurology Grand Rounds
- 2008 St. John's Hospital Neurology Grand Rounds
- 2008 Washington University Neurosurgery Grand Rounds
- 2008 Washington University Hope Center Noon Seminar
- 2008 Washington University Neuroscience Program Seminar
- 2009 Washington University Cell Biology Department Seminar
- 2011 Washington University Hope Center Noon Seminar – Series Organizer and Presenter
- 2012 Washington University Genetics Department Seminar
- 2013 Washington University Hope Center Noon Seminar – Series Organizer and Presenter
- 2013 St. Louis University Brain Awareness Day Symposium
- 2014 Washington University Neurology Grand Rounds
- 2016 Washington University Motor Neuron Lecture to fellows
- 2016 Washington University Neuroscience Seminar Series Presenter
- 2016 Washington University Hope Center Noon Seminar Presenter
- 2017 Washington University Hope Center Noon Seminar Presenter
- 2017 Washington University NRTG Meeting
- 2017 Washington University MCB Systems Neurodegeneration Lecturer
- 2017 Southern Illinois University Lecturer
- 2017 Mercy Hospital Speaker
- 2018 Washington University Hope Center Noon Seminar Presenter
- 2019 Washington University Department of Neurology Grand Rounds Lecturer
- 2020 Washington University MCB Systems Neurodegeneration Lecturer
- 2020 Washington University Neuromuscular Fellow Lecture Series
- 2020 Washington University Markey Course in Dementia, Lecturer
- 2021 Washington University MCB Systems Neurodegeneration Lecture
- 2021 Washington University NM Fellowship Lecture
- 2021 Washington University Genetics and Genomics of Disease Class Lecture
- 2021 Washington University Mini-Medical School Lecture
- 2021 Washington University Hope Center Mini Series Organizer and Lecturer
- 2022 Washington University Journal Club Presenter
- 2022 Washington University Clinical Trials in Neuromuscular Medicine Speaker
- 2022 Washington University Genetics and Genomics of Disease Class Lecturer
- 2022 Washington University Motor Neuron Disease Presenter
- 2023 Washington University NeuroScience Lecture Series
- 2023 Washington University Professor Rounds Speaker
- 2023 Washington University Biochemistry Science Friday Seminar Speaker
- 2023 Washington University Hope Center Mini Series Lecturer
- 2024 Washington University CNS Lecturer
- 2024 Washington University Advancing Neuroscience Seminar Speaker
- 2024 Washington University Big Ideas in Neurology Series Lecturer
- 2024 Washington University Eureka! Science Socials Speaker
- 2024 Washington University NM-Clinical Trials in Neuromuscular Medicine Speaker
- 2024 Saint Louis University Grand Rounds Speaker
- 2024 Washington University Journal Club Presenter
- 2024 Saint Louis University – Institute for Translational Neuroscience Speaker
- 2024 Washington University Neuromuscular Journal Club Presenter
- 2024 Washington University Genetics & Genomics of Disease Lecturer
- 2025 Washington University Neurology Grand Rounds Speaker

2025 Washington University NM Case Series and Grand Rounds Presenter
2025 Washington University Neurology Resident Research Forum Lecturer
2025 Washington University Neuromuscular Clinical Trials Lecturer
2025 Washington University Neuromuscular Journal Club Presenter
2025 Washington University Genetics & Genomics of Disease Lecturer

INDUSTRY RELATIONS:

Research collaboration with Ionis Pharmaceuticals, Inc.
Research collaboration with Biogen Idec, Inc.

CURRENT SUPPORT:

Research Grant Kissick Family Foundation <i>"Developing a Misfolded TDP-43 Molecular Degradar"</i> Role: Co-Principal Investigator	09/01/2025 – 8/30/2027 \$500,000
Research Grant Simons Foundation <i>"A Pilot Platform for Restoring Autism Gene Expression"</i> Role: Co-investigator	02/01/2025-01/31/2027 \$300,000
Research Grant National Institutes of Health <i>"Self-assembling Nanofiber Vaccines for Neurodegeneration"</i> Role: Co-investigator	09/30/2024 – 05/31/2029 \$2,953,388
Research Grant Rainwater Charitable Foundation <i>"Developing 4R Tau-Targeting Antisense Oligonucleotides"</i> Role: Principal Investigator	08/01/2024 – 07/30/2026 \$400,000
Research Grant National Institutes of Health <i>"Directly Converted Neurons as a Novel Cellular Model for ALS and FTD"</i> Role: Principal Investigator	07/01/2024 – 06/30/2026 \$275,000
Research Grant Hope Center for Neurological Disorders <i>"Developing a Molecular Degradar Specific for Misfolded TDP-43"</i> Role: Co-investigator	01/01/2024 – 12/31/2025 \$100,000
Research Grant University of California, San Francisco (subaward to WashU) <i>"Harmonizing remote smartphone assessments of cognition, behavior, and motor functioning across the spectrum of FTD and ALS"</i> Role: Site PI	01/01/2024 – 12/31/2025 \$48,639 (Miller portion)
Research Grant National Institutes of Health (subaward from MGH)	09/25/2023 – 08/31/2027 \$229,725

“Clinical Site Agreement with MGH for NIH Study-An Intermediate-Size Expanded Access Protocol for Amyotrophic Lateral Sclerosis with Pridopidine”

Role: Site PI

Research Grant 09/25/2023 – 09/24/2025
National Institutes of Health (subaward to WashU) \$250,584.00
“Access for All in ALS (ALL ALS) West Clinical Coordinating Center”
Role: Co-investigator

Research Grant 09/22/2023 – 09/21/2025
National Institutes of Health (Subaward to WashU) \$295,000
“ALL ALS West”
Role: Site PI

Research Grant 07/01/2023 – 06/30/2026
Department of Defense \$399,169
“Development of Lovastatin or related compounds as a therapy for ALS”
Role: Co-investigator

Research Grant 08/01/2022 – 07/31/2026
Chan Zuckerberg Initiative \$173,000 per year (Miller)
“Increasing mRNA translation to treat neurodegeneration”
Role: Co-Principal Investigator

Research Grant 11/01/2020 – 10/31/2025
Target ALS \$86,832 per year
“Longitudinal Biofluids Core”
Role: Principal Investigator

Research Grant 01/01/2020 – 12/31/2025
Target ALS \$465,827
“Target ALS Multi-Center Human Post-mortem Tissue Core”
Role: Co-Site PI

PRIOR SUPPORT:

Sponsored Research Agreement 03/01/2022 – 02/28/2025
Ionis Pharmaceuticals, Inc. \$657,658
“Understanding Protein Release from Cells in Neurodegenerative Disease”
Role: Principal Investigator

Research Grant 03/01/2024 – 02/28/2025
University of Missouri – Spinal Cord Injuries/Disease \$100,000
Research Program
“Defining Motor Neuron Vulnerability in Amyotrophic Lateral Sclerosis”
Role: Principal Investigator

Research Grant 08/01/2023 – 07/30/2024
Rainwater Charitable Foundation \$200,000
“Developing 4R Tau-Targeting Antisense Oligonucleotides in Novel Mouse Models of Tauopathy”
Role: Principal Investigator

Research Grant National Institutes of Health <i>"Molecular recording to predict cell fate decisions and animal behavior"</i> Role: Co-investigator	08/01/2021 – 07/30/2024 \$86,061 per year
Research Grant RTW Charitable Foundation <i>"Development of ASOs to restore normal MYT1L Levels"</i> Role: Co-investigator	11/01/2022 – 10/31/2023 \$178,000
Research Grant Packard Center <i>"Using the Answer ALS dataset to expand genetically defined ALS subtypes"</i> Role: Principal Investigator	08/01/2021 – 12/31/2023 \$90,265
Research Grant Target ALS <i>"High Resolution Analysis of Neurofilament in ALS biofluids"</i> Role: Principal Investigator	11/02/2020 – 10/31/2023 \$260,000 (Miller portion)
Research Grant Rainwater Charitable Foundation <i>"Elucidating Mechanisms and Therapeutic Targeting of 4R Tau-induced Toxicity in Astrocytes"</i> Role: Principal Investigator	08/01/2022 – 07/30/2023 \$200,000
Research Grant U.S. Department of Defense <i>"RNA-Based Approach to Decrease TDP-43 Pathology and Neurotoxicity in ALS"</i> Role: Co-Principal Investigator	07/01/2020 – 06/30/2023 \$250,000 (Miller portion)
Research Grant Tau Consortium <i>"Elucidating mechanisms and therapeutic targeting of 4R tau-induced toxicity in astrocytes"</i> Role: Principal Investigator	12/01/2021 – 07/31/2022 \$109,412
Research Grant Hope Center for Neurological Disorders <i>"Identification of Repurposed Prescription Medications as Neuroprotective Therapy for Amyotrophic Lateral Sclerosis"</i> Role: Co-Investigator	07/01/2019 – 06/30/2022 \$50,000 (Miller portion)
Research Grant Chan Zuckerberg Initiative <i>"Using antisense oligonucleotides to increase mRNA translation"</i> Role: Co-Principal Investigator	12/01/2020 – 06/01/2022 \$75,000 (Miller portion)
R01NS078398 National Institutes of Health, NINDS <i>"Using motor neuron specific miRNA to understand and develop novel therapeutics for motor neuron disease."</i>	04/01/2017 – 03/31/2022 \$385,000 per year

Role: Principal Investigator

Research Grant 11/15/2016 – 12/31/2020
ALS Finding a Cure / ALS Association \$999,931
“Generation of a PET Tracer for TDP-43 Aggregates for ALS and FTD”
Role: Principal Investigator

Research Grant 12/01/2020 – 11/30/2021
Rainwater Tau Consortium \$200,000
“Characterizing the impact of increased 4R tau in human astrocytes and their potential as a therapeutic target”
Role: Principal Investigator

1RF1MH11707002 09/01/2018 – 08/31/2021
National Institutes of Health \$769,580
“Parallel Analysis of Transcription and Protein-DNA Interactions in Single CNS Cells”
Role: Co-Investigator

Research Grant 07/01/2018 – 06/30/2021
BrightFocus Foundation \$298,335
“Antisense-mediated TREM2 Knockdown to Lessen Amyloid and Tau Pathology”
Role: Principal Investigator

R01NS097816 06/01/2016 – 05/31/2021
National Institutes of Health, NINDS \$420,000 per year
“Understanding SOD1 Kinetics in Amyotrophic Lateral Sclerosis”
Role: Principal Investigator

Research Grant 01/08/2018 – 01/07/2021
ALS Association \$77,750
“Pathological Analysis of Selective Autophagy in Sporadic and Familial ALS”
Role: Principal Investigator

Research Grant 08/01/2018 – 12/31/2020
ALS Biomarker Consortium \$100,000
“Determining If Motor Neuron-specific miRNA are Biomarkers for Motor Neuron Disease”
Role: Principal Investigator

Research Grant 08/01/2019 – 07/31/2020
Rainwater Tau Consortium \$200,000
“Identifying the impact of increased 4R tau in human astrocytes and developing astrocyte targeted therapeutics”
Role: Principal Investigator

Research Grant 08/01/2017 – 07/31/2020
Muscular Dystrophy Association \$94,000 per year
“Understanding RAN Dipeptide Size and Kinetics in C9orf72 ALS”
Role: Principal Investigator

Research Grant 05/31/2018 – 05/31/2020
Missouri Spinal Cord Injury/Disease Research Program \$250,000

“Understanding Normal and Aberrant miRNA-mediated Motor Neuron Astrocyte Communication”
Role: Principal Investigator

Research Grant 09/01/2016 – 07/31/2022
ALS Finding a Cure / ALS Association \$100,000
“Dominantly Inherited ALS Network (DIALS)”
Role: Site Principal Investigator

Research Grant 09/01/2015 – 03/31/2020
ALS Association \$65,000 per year
“Genomics Translation for ALS Clinical Care (GTAC)”
Role: Site Principal Investigator

Research Grant 07/01/2015 – 03/31/2020
Packard Center / ALS Finding a Cure \$70,000 per year
“Answer ALS”
Role: Site Investigator

Research Grant 07/01/2019 – 12/31/2019
Target ALS \$50,830
“Post Mortem Tissue Core”
Role: Principal Investigator

Research Grant 05/31/2018 – 11/30/2019
Rainwater Tau Consortium \$200,000
“Targeting Tau in Astrocytes to Modulate Pathology and Neurodegeneration”
Role: Principal Investigator

U01MH10913301 09/18/2015 – 6/30/2019
National Institutes of Health, NIMH \$53,000 per year
“An Inducible Molecular Memory System to Record Transient States of CNS Cells”
Role: Co-Investigator (Rob Mitra, Co-PI)

Research Grant 09/19/2015 – 11/01/2018
ALS Hub – Biogen / Quintiles Transnational, Inc. \$17,000 per year
“Methodology Study of Novel Outcome Measures to Assess the Progression of ALS”
Role: Site Investigator

Research Grant 07/31/2016 – 09/30/2018
ALS Finding a Cure / ALS Association \$250,000
“Understanding SOD1 Kinetics in Amyotrophic Lateral Sclerosis”
Role: Principal Investigator

R21NS099766 09/01/2016 - 08/31/2018
National Institutes of Health, NINDS \$74,674
“Endogenous Peptides as Markers of Abnormal SOD-1 Protein in Familial and Sporadic ALS”
Role: Collaborator

Research Grant 08/01/2017 – 07/31/2018
Rainwater Tau Consortium \$200,000
“Determining the Link Between Tau Isoform Changes and Astrocyte Pathology”

Role: Principal Investigator

Research Grant 09/01/2015 – 07/31/2018
ALS Association / ALS Finding a Cure \$27,000 per year
“Cross Sectional ALS Biofluid Biomarker Study (CABB)”
Role: Site Investigator

Research Grant 11/01/2014 – 07/31/2018
ALS Association \$170,000 per year
“Understanding Clinical Phenotype and Collecting Biomarker Samples in C9ORF72 ALS”
Role: Principal Investigator

Sponsored Research Agreement 09/15/2013 – 12/31/2017
Biogen Idec, Inc. \$300,000 per year
“Natural History and Biomarkers for C9ORF72 ALS”
Role: Principal Investigator

Research Grant 09/01/2016 – 08/31/2017
Rainwater Charitable Foundation \$150,000
“Developing Antisense Oligonucleotide Therapeutics for Tau”
Role: Principal Investigator

Research Grant 05/01/2016 – 04/30/2017
Packard Center at Johns Hopkins \$80,000
“miRNAs enriched in ALS-vulnerable motor neurons as therapeutic targets”
Role: Principal Investigator

R01NS078398 04/15/2012 – 02/29/2017
National Institutes of Health, NINDS \$330,000 per year
“Developing a MicroRNA-Targeted Therapy for ALS”
Role: Principal Investigator

Research Grant 07/01/2014 – 12/31/2016
McDonnell Center for Cellular and Molecular Neurobiology \$20,000 per year
“F18 T807 tau PET imaging in familial amyotrophic lateral sclerosis”
Role: Principal Investigator

U01NS08497002 04/01/2014 – 08/31/2016
National Institutes of Health, NINDS \$330,000 per year
“Development of an Antisense Oligonucleotide Therapy for SOD1 Familial ALS”
Role: Principal Investigator

Research Grant 07/01/2014 – 06/30/2016
University of Missouri SCIDRP \$250,000 per year
“Comprehensive miRNA Analysis of Specific Cell Types in Spinal Cord”
Role: Principal Investigator

Research Grant 07/01/2015 – 06/31/2016
Rainwater Charitable Foundation \$160,000
“Decreasing Tau Protein as a Therapy for Tauopathies”
Role: Principal Investigator

R21AG0447190 National Institutes of Health, NIA <i>"Does a Shift from 4R TO 3R Tau Project Against Amyloid Beta-Induced Cognitive deficits?"</i> Role: Principal Investigator	07/01/2013 – 06/30/2016 \$275,000 per year
Sponsored Research Agreement Ionis Pharmaceuticals, Inc. <i>"Developing Antisense Oligonucleotides that Target Tau"</i> Role: Principal Investigator	02/24/2014 – 02/23/2016 \$330,000 per year
P50AG005681 National Institutes of Health, NIA <i>"Alzheimer's Disease Research Center: Project 2"</i> Role: Principal Investigator	05/01/2010 – 04/30/2015 \$105,000 per year
Research Grant Target ALS / Columbia University <i>"Targeting miR-155 with antisense oligonucleotides as a therapy for ALS"</i> Role: Principal Investigator	10/15/2013 – 10/14/2015 \$200,000 per year
Research Grant ALS Association <i>"Determining the Half-Life of SOD1 in Human CSF"</i> Role: Principal Investigator	08/01/2013 – 12/31/2014 \$140,000
Research Grant Tau Consortium <i>"Decreasing Tau mRNA and Protein as a Therapy for Tauopathies"</i> Role: Principal Investigator	07/29/2011 – 07/28/2014 \$150,000 per year
R21NS078242/sub #C00038097-1 National Institutes of Health, University of Missouri <i>"Therapeutic Development for Amyotrophic Lateral Sclerosis in a Canine Model"</i> Role: Collaborator (Joan Coates, PI)	12/01/2012 – 11/30/2014 \$18,000 per year
Research Grant Muscular Dystrophy Association <i>"The Natural History of SOD1 ALS"</i> Role: Principal Investigator	07/01/2012 – 12/31/2014 \$225,000 per year
K08NS074194 National Institutes of Health, NINDS/AFAR <i>"Changing tau Protein Levels and tau Protein Isoforms in Mouse Models of Dementia"</i> Role: Principal Investigator	09/30/2010 – 08/31/2013 \$220,000 per year
R21NS072584 National Institutes of Health, NINDS <i>"Identifying Liver Proteins that Decrease mutant SOD1 Misfolding and Decrease SOD1 Toxicity"</i> Role: Principal Investigator	09/01/2010 – 08/31/2012 \$225,000 per year
Research Grant	04/01/2007 – 12/31/2012

Muscular Dystrophy Association \$315,000 per year
"Toxicology and Phase I Studies of Antisense ISIS 333611 in Familial ALS"
 Role: Principal Investigator

Research Grant 06/30/2011 – 06/29/2014
 Packard Center for ALS Research \$60,000 per year
"Understanding miR-155 in ALS"
 Role: Principal Investigator

Alzheimer's Disease Research Center Pilot Grant 06/01/2009 – 05/31/2010
 National Institutes of Health, NIA \$35,000
"Decreasing tau protein levels and decreasing tau 4R:3R isoforms in mouse models of dementia"
 Role: Principal Investigator

Research Grant 06/01/2009 – 05/31/2010
 Washington University ICTS \$25,000
"Measuring SOD1 in the Cerebral Spinal Fluid of ALS Patients"
 Role: Principal Investigator

Research Grant 06/01/2010 – 05/31/2011
 Washington University ICTS \$25,000
"Does SMN copy number influence survival in Amyotrophic Lateral Sclerosis?"
 Role: Principal Investigator

Research Grant 10/01/2009 – 09/30/2012
 Edward Mallinckrodt, Jr. Foundation \$60,000 per year
"Understanding MicroRNA Changes in Amyotrophic Lateral Sclerosis"
 Role: Principal Investigator

Sponsored Research Agreement 06/12/2009 – 06/22/2011
 Ionis Pharmaceuticals, Inc. \$70,000 per year
"Development of Therapeutic Strategies for ALS and Dementia"
 Role: Principal Investigator

Research Grant 07/01/2011-06/30/2012
 CurePSP Foundation \$75,000
"Evaluating Reduced Tau Levels as a Therapy for PSP and CBD"
 Role: Principal Investigator

Research Grant 02/01/2008 – 01/31/2009
 ALS Association \$40,000
"Downregulation of SOD1 in the CSF of SOD1G93A Rats Using Antisense Oligonucleotides"
 Role: Principal Investigator

PATENTS:

<u>Docket Number</u>	<u>Filing Date</u>	<u>Application Type</u>	<u>Patent Title</u>
011873-PRO1	10/17/2011	Provisional	Metabolism of SOD1 in CSF

011873-PCT1/1	10/17/2012	PCT	Turnover rate and half life of Superoxide dismutase 1 (SOD1) in human cerebrospinal fluid (CSF)
012145-PRO1	3/6/2012	Provisional	Method of diagnosing neurodegenerative diseases with microRNA detection
012145-PRO2/1	3/6/2012	Provisional	Method of treating neurodegenerative diseases with microRNA regulators
012145-PRO3/1	12/4/2012	Provisional	Method for widespread miR-155 inhibition prolongs survival in ALS mice
012145-PCT1/1	3/6/2013	PCT	Method of treating neurodegenerative diseases with microRNA regulators
012147-PRO1	3/30/2012	Provisional	Compositions and methods of modifying a neurodegenerative syndrome
012147-PRO2	6/15/2012	Provisional	Compositions and methods of reducing seizure and modifying a neurodegenerative syndrome
012147-PRO3	10/26/2012	Provisional	Methods for modulating Tau expression
012147-PCT1/1	3/14/2013	PCT	Methods for modulating Tau expression for reducing neurodegenerative syndrome
012147-AU01/1	3/14/2013	FOR - Foreign	Changing Tau splicing as a therapy for neurological disorders

CLINICAL RESPONSIBILITIES:

Attending Physician in the Washington University Neuromuscular Division/MDA clinic. I attend one half day a week in the neuromuscular/MDA clinic at Washington University. I care for patients with neuromuscular disorders including muscular dystrophy, myositis, myotonia, ALS, SMA, immune-mediated neuropathies, inherited neuropathies, ataxias and spastic paraparesis. This involves primary patient care and supervision of medical students, residents, and fellows. Consult Service Ward Attending one month out of the year at Barnes-Jewish Hospital. This involves supervision of residents and medical students.

UNIVERSITY/DEPARTMENTAL SERVICE:

Lecturer, Neurobiology of Disease Neurosciences Course, 2008 - present
Course Instructor for Molecular and Cellular Neuroscience Course for first-year graduate students (teach four 1.5-hour lectures), 2009 – 2017
Neuroscience Admissions Committee, 2009 – 2014
Lecturer, Neuromuscular Fellows, 2009 - present
Lecturer, Neurobiology of Disease, Genetics Course, 2010 - present
Lecturer, Neurobiology of Disease, Cell Biology Program Course, 2012 - present
Neurology Residency Board Exam Reviewer, 2014 – present
Neurology Resident Mentorship Committee, 2016 - present

Member, Search Committee for Washington University Attending Veterinarian, 2017
 Knight ADRC T32 Fellowship Committee, 2018 - present
 Medical Scientist Training Program Admissions Committee, 2018 – present
 Clinical Competency Committee/Program Evaluation Committee (CCC/PEC), 2019 - present
 Judge, Resident Research Symposium, 2020
 Member, Committee on Research Integrity, 2021 – present

THESIS COMMITTEES:

2007-2009 Member: Katherine Schwetye, Lab of David Brody
 2008-2011 Chair: Iga Wegorzewska, Lab of Bob Baloh
 2008-2010 Member: Albert Misko, Lab of Bob Baloh
 2008-2011 Member: Joseph Castellano, Lab of David Holtzman
 2010-2012 Member: Adreu Viader, Lab of Jeff Milbrandt
 2012-2015 Member: Jeung Eun Shin, Lab of Aaron DiAntonio
 2013-2014 Member: Yasuko Suzuki, Lab of Paul Kotzbauer
 2009-2014 Member: Donell Carey, Lab of Heather True-Krob
 2010-2014 Chair: Janet Cady, Lab of Matthew Harms
 2013-2018 Member: Tirth Patel, Lab of Dave Holtzman
 2014-2017 Member: Sarah Kaufman, Lab of Marc Diamond
 2014-2018 Member: Cheryl Leyns, Lab of Dave Holtzman
 2014-2019 Chair: Sarah Smith, Lab of Azad Bonni
 2014-2018 Chair: Eugene Lee, Lab of Conrad Wehl
 2014-2018 Member: Tien-Phat (Phat) Huynh, Lab of Dave Holtzman
 2017-2018 Member: Sharma Apurwa, Lab of Mark Diamond
 2017-2022 Chair: Jiang Zhu, Lab of Conrad Wehl
 2018-2019 Member: Umber Dube, Lab of Brad Racette
 2019-2020 Member: Percy Griffin, Lab of Erik Musiek
 2020-2023 Member: Caitlin Dingwall, Lab of Jeff Milbrandt
 2020-2021 Member: Thomas Mahan, Lab of Dave Holtzman
 2021-2023 Member: Kelsey Krus, Lab of Aaron DiAntonio
 2021-2023 Member: Garret Ginell, Lab of Alex Holehouse
 2022-present Member: Sarah Koester, Lab of Joseph Dougherty
 2022-present Chair: Collin Nadarajah, Lab of Erik Musiek
 2023-present Chair: Noah Basri, Lab of Ting Wang
 2023-present Member: Sarah Schurr, Lab of Aaron DiAntonio
 2023-present Member: Mari Gachechiladze, Lab of Joseph Dougherty
 2024-present Member: Darcy Harris, Lab of Meridith Jackrel and Jai Rudra
 2025-present Member: Wendy Dong, Lab of Jeffrey Milbrandt
 2025-present Member: Cameron Ortiz, Lab of Chris Wehl
 2025-present Member: Kylie Schache, Lab of Celeste Karch
 2025-present Member: Joey Zemke, Lab of Celeste Karch
 2025-present Member: Lloyd Tripp, Lab of Rob Mitra

POSTDOCTORAL FELLOWS SUPERVISED:

Year	Name	School/Position During Training	Role During Lab Rotation	Current Position
2007-2010	Quan Li, PhD	Post-doctoral fellow	Faculty mentor	Research Scientist, Osaka University

2012-2013	Georgios Manousakis, MD	Post-doctoral fellow	Faculty mentor	Associate Professor, Univ. of Minnesota
2013-2015	Taha Bali, MD	Post-doctoral fellow	Faculty mentor	Assistant Professor, Tufts University
2015-2016	Linga Reddy, MD	Post-doctoral fellow	Faculty mentor	Neurologist, First Choice Neurology
2013-2017	Kathleen Schoch, PhD	Post-doctoral fellow	Faculty mentor	Assistant Professor, Washington University
2015-2018	Cindy Ly, MD, PhD	Post-doctoral fellow	Faculty mentor	Assistant Professor, Washington University
2020-2024	Pranav Vemula, PhD	Post-doctoral fellow	Faculty mentor	Assistant Professor, Washington University
2020-2022	Ishita Parikh, PhD	Post-doctoral fellow	Faculty mentor	Private Industry (Scientific)
2020-2022	Collin Kreple, MD, PhD	Post-doctoral fellow	Faculty mentor	Assistant Professor, University of Wisconsin
Jan 2021- May 2021	Gabriel Vazquez Velez, MD, PhD	Post-doctoral fellow	Faculty mentor	Postdoctoral Fellow/Resident, Washington University
August 2021- Present	Dylan Galloway, PhD	Post-doctoral fellow	Faculty mentor	Postdoctoral Scholar
February 2023 - Present	Ravi Chopra, MD, PhD	Adult Neurology Resident	Faculty co-mentor R25	Adult Neurology Resident
August 2023 - Present	Zita Hubler, MD, PhD	Post-doctoral fellow	Faculty mentor	Postdoctoral Scholar
April 2025 - Present	Max Liu, MD, PhD	Post-doctoral fellow	Faculty mentor	Postdoctoral Scholar

PREDOCTORAL FELLOWS SUPERVISED:

Year	Name	School/Position During Training	Role During Lab Rotation	Current Position
2008-2014	Erica Koval	Pre-doctoral fellow	Faculty mentor	Principal Scientist, Biogen Idec
2008-2014	Sarah DeVos	Pre-doctoral fellow	Faculty mentor	Senior Director, Curie Bio

2009-2014	Matthew Crisp	Pre-doctoral fellow	Faculty mentor	Hospitalist, Mercy Hospital Jefferson, Festus, MO
2013-2018	Mariah Lawler Hoyer	Pre-doctoral fellow	Faculty mentor	Health Program Specialist, NIH
2014-2018	Wade Self	Pre-doctoral fellow	Faculty mentor	Instructor, Washington University
2015-2020	Alex Cammack	Pre-doctoral fellow	Faculty mentor	Postdoctoral Fellow, UCL Queen Square Institute of Neurology, London, UK
2016-2021	Lubov Ezerskiy	Pre-doctoral fellow	Faculty mentor	Postdoctoral Fellow, University of Texas
2018-2021	Nathan Pomper	Pre-doctoral fellow	Faculty mentor	Industry
2018-2023	Hunter Patterson	Pre-doctoral fellow	Faculty mentor	Medical/Graduate Student
2020-2025	Ben Boros	Pre-doctoral fellow	Faculty mentor	Medical/Graduate Student
2021-present	Miwei Hu	Pre-doctoral fellow	Faculty mentor	Graduate Student
2022-present	Grace Cooper	Pre-doctoral fellow	Faculty mentor	Graduate Student
2024-present	Eunji Chang	Pre-doctoral fellow	Faculty mentor	Graduate Student
2024-present	Aishwarya Nambiar	Pre-doctoral fellow	Faculty mentor	Graduate Student
2025-present	Paige Kirschner	Pre-doctoral fellow	Faculty mentor	Graduate Student

KEYWORDS/AREAS OF INTEREST:

ALS, Neurodegeneration, SOD1, Tau, antisense oligonucleotide, miRNA

PUBLICATIONS:

ORIGINAL RESEARCH ARTICLES

1. Krivickas B, Scirocco E, Giacomelli E, Sharma S, Benson M, Keegan M, Kulesa-Kelley J, Chibnik LB, Casagrande G, Heyd L, Chase M, Drake K, Mohapatra S, Hagar JL, Hasenoehrl MG, Dagostino D, Sherman AV, Leite A, Yu H, Rosenthal J, **Miller T**, McCaffrey A, Gwathmey K, Locatelli E, Bayat E, Heitzman D, Young E, Goyal NA, Whitesell J, Felice K, Ilieva H,

- Swenson A, Walk D, Alameda G, Foster L, McIllduff CE, Walsh A, Zilliox L, Ajroud-Driss S, Bodkin C, Katz J, Ladha S, Rivner M, Rosow L, Tywdell P, Wasiewski W, Babu S, Berry JD, Paganoni S. Multicenter Expanded Access Protocol for Research Through Access to Trehalose in People with Amyotrophic Lateral Sclerosis. *Muscle Nerve*. **2025** Aug 26:10.1002/mus.70011. doi: 10.1002/mus.70011. Online ahead of print. PMID: 40857020.
2. Rothstein JD, Keeley O, Warlick C, **Miller TM**, Ly CV, Glass JD, Coyne AN. Sporadic ALS induced pluripotent stem cell derived neurons reveal hallmarks of TDP-43 loss of function. *Nat Commun*. **2025** Aug 2;16(1):7092. doi: 10.1038/s41467-025-62482-7. PMID: 40753166.
 3. Shefner JM, Cudkowicz ME, Genge A, Hardiman O, Al-Chalabi A, Andrews J, Chio A, Corcia P, Couratier P, de Carvalho M, Heiman-Patterson T, Henderson RD, Ingre C, Johnston W, Ludolph A, Maragakis NJ, **Miller TM**, Mora JS, Petri S, Simmons Z, van den Berg LH, Zinman L, Kupfer S, Malik FI, Meng L, Simkins TJ, Wei J, Wolff AA, Rudnicki SA. Reldesemtiv in Amyotrophic Lateral Sclerosis: Results from the COURAGE-ALS Randomized Clinical Trial. *JAMA Neurol*. **2025** May 1;82(5):477-485. doi: 10.1001/jamaneurol.2025.0241. PMID: 40126464.
 4. Smith SE, McCoy-Gross K, Malcolm A, Oranski J, Markway JW, **Miller TM**, Bucelli RC. Tofersen treatment leads to sustained stabilization of disease in SOD1 ALS in a “real-world” setting. *Ann Clin Transl Neurol*. **2025** Feb;12(2):311-319. Doi: 10.1002/acn3.52264. Epub 2025 Jan 9. PMID: 39783194.
 5. Sumner CJ, **Miller TM**. The expanding application of antisense oligonucleotides to neurodegenerative diseases. *J Clin Invest*. **2024**;134(19):e186116. doi.org/10.1172/JCI186116.
 6. Berry JD, Paganoni S, Harms MB, Shneider N, Andrews J, **Miller TM**, Babu S, Sherman AV, Harris BT, Provenzano FA, Phatnani HP, Shefner J, Garret MA, Ladha SS, Tsou AY, Mohan P, Ingre C, ALL ALS Consortium, Bowser R. Access for ALL in ALS: A large-scale, inclusive, collaborative consortium to unlock the molecular and genetic mechanisms of amyotrophic lateral sclerosis. *Muscle Nerve*. **2024** Sep 18. doi: 10.1002/mus.28244. Online ahead of print. PMID: 39295118.
 7. Trivedi RR, Archambault AS, Pavlak C, Gastaldi M, Cantoni C, Ghezzi L, Cross AH, **Miller TM**, Wu GF. Prevalence of anti-myelin oligodendrocyte glycoprotein antibodies across neuroinflammatory and neurodegenerative diseases. *J Neurol Sci*. **2024** Jun 15:461:123041. doi: 10.1016/j.jns.2024.123041. PMID: 38744216.
 8. Irwin, KE, Jasin P, Braunstein KE, Sinha IR, Garret M, Bowden KD, Chang K, Troncoso JC, Moghekar A, Oh ES, Raitcheva D, Bartlett D, **Miller T**, Berry JD, Traynor BJ, Ling JP, Wong, PC. A fluid biomarker reveals loss of TDP-43 splicing repression in presymptomatic ALS-FTD. *Nat Med*. **2024** Feb;30(2):382-393. doi: 10.1038/s41591-023-02788-5. PMID: 38278991.
 9. Iyer AK, Schoch KM, Verbeck A, Galasso G, Chen H, Smith S, Oldenburg A, **Miller TM**, Karch CM, Bonni A. Targeted ASO-mediated Atp1a2 knockdown in astrocytes reduces SOD1 aggregation and accelerates disease onset in mutant SOD1 mice. *PLoS One*. **2023** Nov 28;18(11). doi: 10.1371/journal.pone.0294731. PMID: 38015828.
 10. Vemula P, Schoch KM, **Miller TM**. Evaluating the efficacy of purchased antisense oligonucleotides to reduce mouse and human tau in vivo. *Front. Mol. Neurosci*. 2023 Dec 18;16. doi: 10.3389/fnmol.2023.1320182. PMID: 38192302.
 11. Kreple CJ, Gajagowni S, Jockel-Balsarotti J, Bucelli RC, **Miller TM**. Lumbar punctures are safe in patients with ALS and have a similar risk profile relative to the non-ALS population. *Muscle Nerve*. **2023** Nov;68(5):771-775. doi: 10.1002/mus.27956. Epub 2023 Aug 11. PMID: 37566385.
 12. Shefner JM, Al-Chalabi A, Andrews JA, Chio A, De Carvalho M, Cockroft BM, Corcia P, Couratier P, Cudkowicz ME, Genge A, Hardiman O, Heiman-Patterson T, Henderson RD, Ingre C, Jackson CE, Johnston W, Lechtzin N, Ludolph A, Maragakis NJ, **Miller TM**, Mora Pardina JS, Petri S, Simmons Z, Van Den Berg LH, Zinman L, Kupfer S, Malik FI, Meng L, Simkins TJ, Wei J, Wolff AA, Rudnicki SA. COURAGE-ALS: a randomized, double-blind phase 3 study

- designed to improve participant experience and increase the probability of success. *Amyotroph Lateral Scler Frontotemporal Degener.* **2023** Aug;24:5-6, 523-534. doi: 10.1080/21678421.2023.2216223. PMID: 37254449.
13. Sun R, Han R, McCornack C, Khan S, Tabor GT, Chen Y, Hou J, Jiang H, Schoch KM, Mao DD, Cleary R, Yang A, Liu Q, Luo J, Petti A, **Miller TM**, Ulrich JD, Holtzman DM, Kim AH. TREM2 inhibition triggers antitumor cell activity of myeloid cells in glioblastoma. *Sci Adv.* **2023** May 12;9(19):eade3559. doi: 10.1126/sciadv.ade3559. Epub 2023 May 12. PMID: 37172094.
 14. Kreple CJ, Searles Nielsen S, Schoch KM, Shen T, Shabsovich M, Song Y, Racette BA, **Miller TM**. Protective effects of lovastatin in a population-based ALS study and mouse model. *Ann. Neurol.* Epub ahead of print **2023** Jan 10. doi: 10.1002/ana.26600. PMID: 36627836.
 15. Gebrehiwet P, Meng L, Rudnicki SA, Sarocco P, Wei J, Wolff AA, Chio A, Andrews JA, Genge A, Jackson CE, Lechtzin N, **Miller TM**, Shefner JM. MiToS and King's staging as clinical outcome measures in ALS: a retrospective analysis of the FORTITUDE-ALS trial. *Amyotroph Lateral Scler Frontotemporal Degener.* **2022** Dec 12:1-7. doi: 10.1080/21678421.2022.2154678. PMID: 36503310.
 16. Shefner JM, Bedlack R, Andrews JA, Berry JD, Bowser R, Brown R, Glass JD, Maragakis NJ, **Miller TM**, Rothstein JD, Cudkowicz ME. Amyotrophic Lateral Sclerosis Clinical Trials and Interpretation of Functional End Points and Fluid Biomarkers: A Review. *JAMA Neurol.* **2022** Dec 1;79(12):1312-1318. doi: 10.1001/jamaneurol.2022.3282. PMID: 36251310.
 17. Opie-Martin S, Iacoangeli A, Topp SD, Abel O, Mayl K, Mehta PR, Shatunov A, Fogh I, Bowles H, Limbachiya N, Spargo TP, Al-Khleifat A, Williams KL, Jockel-Balsarotti J, Bali T, Self W, Henden L, Nicholson GA, Ticozzi N, McKenna-Yasek D, Tang L, Shaw PJ, Chio A, Ludolph A, Weishaupt JH, Landers JE, Glass JD, Mora JS, Robberecht W, Van Damme P, McLaughlin R, Hardiman O, van den Berg L, Veldink JH, Corcia P, Stevic Z, Siddique N, Silani V, Blair IP, Fan D, Esselin F, de la Cruz E, Camu W, Basak NA, Siddique T, **Miller T**, Brown RH, Al-Chalabi A, Shaw CE. The SOD1-mediated ALS phenotype shows a decoupling between age of symptom onset and disease duration. *Nat Commun.* **2022** Nov 12;13(1):6901. Doi: 10.1038/s41467-022-34620-y. PMID: 36371497.
 18. **Miller TM**, Cudkowicz ME, Genge A, Shaw PJ, Sobue G, Bucelli RC, Chiò A, Van Damme P, Ludolph AC, Glass JD, Andrews JA, Babu S, Benatar M, McDermott CJ, Cochrane T, Chary S, Chew S, Zhu H, Wu F, Nestorov I, Graham D, Sun P, McNeill M, Fanning L, Ferguson TA, Fradette S. Trial of Antisense Oligonucleotide Tofersen for *SOD1* ALS. *N Engl J Med.* **2022** Sep 22;387(12):1099-1110. doi: 10.1056/NEJMoa2204705. PubMed PMID: 36129998
 19. Boros BD, Schoch KM, Kreple CJ, **Miller TM**. Antisense Oligonucleotides for the Study and Treatment of ALS. *Neurotherapeutics.* **2022** Jun 2. doi: 10.1007/s13311-022-01247-Review. PMID: 35653060.
 20. Camacho-Soto A, Searles Nielsen S, Faust IM, Bucelli RC, **Miller TM**, Racette BA. Incidence of amyotrophic lateral sclerosis in older adults. *Muscle Nerve.* **2022** Sep;66(3):289-296. Doi: 10.1002/mus.27652. PMID: 35678083.
 21. Krishnan G, Raitcheva D, Bartlett D, Prudencio M, McKenna-Yasek DM, Douthwright C, Oskarsson BE, Ladha S, King OD, Barmada SJ, **Miller TM**, Bowser R, Watts JK, Petrucelli L, Brown RH, Kankel MW, Gao FB. Poly(GR) and poly(GA) in cerebrospinal fluid as potential biomarkers for C9ORF72-ALS/FTD. *Nat Commun.* **2022** May 19;13(1):2799. doi: 10.1038/s41467-022-30387-4. PMID: 35589711.
 22. Paganoni S, Hendrix S, Dickson SP, Knowlton N, Berry JD, Elliott MA, Maiser S, Karam C, Caress JB, Owegi MA, Quick A, Wymer J, Goutman SA, Heitzman D, Heiman-Patterson TD, Jackson C, Quinn C, Rothstein JD, Kasarskis EJ, Katz J, Jenkins L, Ladha SS, **Miller TM**, Scelsa SN, Vu TH, Fournier C, Johnson KM, Swenson A, Goyal N, Pattee GL, Babu S, Chase M, Dagostino D, Hall M, Kittle G, Eydinov M, Ostrow J, Pothier L, Randall R, Shefner JM, Sherman AV, Tustison E, Vigneswaran P, Yu H, Cohen J, Klee J, Tanzi R, Gilbert W, Yeramian P, Cudkowicz M. Effect of sodium phenylbutyrate/taurursodiol on tracheostomy/ventilation-free

- survival and hospitalisation in amyotrophic lateral sclerosis: long-term results from the CENTAUR trial. *J Neurol Neurosurg Psychiatry*. **2022** May 16;. doi: 10.1136/jnnp-2022-329024. PMID: 35577511.
23. Rudnicki SA, Andrews JA, Genge A, Jackson C, Lechtzin N, **Miller TM**, Cockroft BM, Malik FI, Meng L, Wei J, Wolff AA, Shefner JM; FORTITUDE-ALS STUDY GROUP. Prescription and acceptance of durable medical equipment in FORTITUDE-ALS, a study of *reldesemtiv* in ALS: post hoc analyses of a randomized, double-blind, placebo-controlled clinical trial. *Amyotroph Lateral Scler Frontotemporal Degener*. **2022** May;23(3-4):263-270. doi: 10.1080/21678421.2021.1946083. Epub 2021. PMID: 34218726.
 24. Beukenhorst AL, Burke KM, Scheier Z, **Miller TM**, Paganoni S, Keegan M, Collins E, Connaghan KP, Tay A, Chan J, Berry JD, Onnela JP. Using Smartphones to Reduce Research Burden in a Neurodegenerative Population and Assessing Participant Adherence: A Randomized Clinical Trial and Two Observational Studies. *JMIR Mhealth Uhealth*. **2022** Feb 4;10(2):e31877. doi: 10.2196/31877. PMID: 35119373.
 25. Yim AKY, Wang PL, Bermingham JR Jr, Hackett A, Strickland A, **Miller TM**, Ly C, Mitra RD, Milbrandt J. Disentangling glial diversity in peripheral nerves at single-nuclei resolution. *Nat Neurosci*. **2022** Feb;25(2):238-251. doi: 10.1038/s41593-021-01005-1. PMID: 35115729.
 26. Ezerskiy LA, Schoch KM, Sato C, Beltcheva M, Horie K, Rigo F, Martynowicz R, Karch CM, Bateman RJ, **Miller TM**. Astrocytic 4R tau expression drives astrocyte reactivity and dysfunction. *JCI Insight*. **2022** Jan 11;7(1):e152012. PMID: 34874917.
 27. Grese ZR, Bastos AC, Mamede LD, French RL, **Miller TM**, Ayala YM. Specific RNA interactions promote TDP-43 multivalent phase separation and maintain liquid properties. *EMBO Rep*. **2021** Dec 6;22(12):e53632. doi: 10.15252/embr.202153632. PMID: 34787357.
 28. Alquezar C, Schoch KM, Geier EG, Ramos EM, Scrivo A, Li KH, Argouarch AR, Mlynarski EE, Dombroski B, DeTure M, Dickson DW, Yokoyama JS, Cuervo AM, Burlingame AL, Schellenberg GD, **Miller TM**, Miller BL, Kao AW. TSC1 loss increases risk for tauopathy by inducing tau acetylation and preventing tau clearance via chaperone-mediated autophagy. *Sci Adv*. **2021** Nov 5;7(45):eabg3897. doi: 10.1126/sciadv.abg3897. PMID: 34739309.
 29. Schoch KM, Ezerskiy LA, Morhaus MM, Bannon RN, Sauerbeck AD, Shabsovich M, Paymaan J, Rigo F, **Miller TM**. Acute Trem2 reduction triggers increased microglial phagocytosis, slowing amyloid deposition in mice. *PNAS*. **2021** Jul 6;118(27). PMID: 34187891.
 30. Shefner JM, Andrews JA, Genge A, Jackson C, Lechtzin N, **Miller TM**, et al. A Phase 2, Double-Blind, Randomized, Dose-Ranging Trial of Reldesemtiv in Patients with ALS. *Amyotroph Lateral Scler Frontotemporal Degener*. **2021** May;22(3-4):287-299. PMID: 32969758.
 31. Kreple, CJ, Schoch KM, **Miller TM**. Is presymptomatic ALS perivascular? *Nat Med*. **2021** Apr;27(4):585-586. PMID: 33859434.
 32. Beukenhorst AL, Collins E, Burke KM, Rahman SM, Clapp M, Konanki SC, Paganoni S, **Miller TM**, Chan J, Onnela JP, Berry JD. Smartphone data during the COVID-19 pandemic can quantify behavioral changes in people with ALS. *Muscle Nerve*. **2021** Feb;63(2):258-262. PMID: 33118628.
 33. Paganoni S, Hendrix S, Dickson SP, Knowlton N, Macklin EA, Berry JD, Elliott MA, Maiser S, Karam C, Caress JB, Owegi MA, Quick A, Wymer J, Goutman SA, Heitzman D, Heiman-Patterson TD, Jackson CE, Quinn C, Rothstein JD, Kasarskis EJ, Katz J, Jenkins L, Ladha S, **Miller TM**, Scelsa SN, Vu TH, Fournier CN, Glass JD, Johnson KM, Swenson A, Goyal NA, Pattee GL, Andres PL, Babu S, Chase M, Dagostino D, Hall M, Kittle G, Eydinov M, McGovern M, Ostrow J, Pothier L, Randall R, Shefner JM, Sherman AV, St Pierre ME, Tustison E, Vigneswaran P, Walker J, Yu H, Chan J, Wittes J, Yu ZF, Cohen J, Klee J, Leslie K, Tanzi RE, Gilbert W, Yeramian PD, Schoenfeld D, Cudkowicz ME. Long-term survival of participants in the CENTAUR trial of sodium phenylbutyrate-taurursodiol in amyotrophic lateral sclerosis.

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34. Gendron TF, Badi MK, Heckman MG, Jansen-West KR, Vilanilam GK, Johnson PW, Burch AR, Walton RL, Ross OA, Brott TG, **Miller TM**, Berry JD, Nicholson KA, Wszolek ZK, Oskarsson BE, Sheth KN, Sansing LH, Falcone GJ, Cucchiara BL, Meschia JF, Petrucelli L. Plasma neurofilament light predicts mortality in patients with stroke. *Sci Transl Med.* **2020** Nov 11;12(569). doi: 10.1126/scitranslmed.aay1913. PubMed PMID: 33177179.
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