Abstracts from the 18th Annual NEALS Meeting
Poster Presentations

Poster #:

Category: Basic Science

1. Approaches to potential ALS therapeutics through retinoic acid receptor-modulators showing dual-acting genomic and non-genomic activities.

Thabat Khatib1, Pietro Marinari1, Sudheer Nuuna1, David Chisholm2, Andrew Whiting2, Christopher Redfern3, Iain Grieg1, Peter McCaffery1
1Aberdeen University, Aberdeen, United Kingdom. 2Durham University, Durham, United Kingdom. 3Newcastle University, Newcastle upon Tyne, United Kingdom

2. TP73 functions in amyotrophic lateral sclerosis pathology

Kristi Russell1, Jonathan Downie1, Summer Gibson1, Spyridoula Tsetsou2, Matthew Keefe1, K. Figueroa1, M.B. Bromberg1, L. Charles Murtaugh1, Stefan Pulst1, Josh Bonkowsky1, Lynn Jorde1
1University of Utah, Salt Lake City, USA. 2Mount Sinai Hospital, New York, USA

3. Genetic variants and alterations in WWOX lead to tau phosphorylation and mis-localization in post-mortem ALS motor cortex

Ghazaleh Sadri-Vakili1, Tiziana Petrozziello1, Alexandra Mills1, Sali Farhan2, Kaly Mueller2, Simon Dujardin2, Ana Amaral2, Teresa Gomez-Isla2, Bradley Hyman2, Khashayar Vakili3
1Healey Center for ALS, MGH, Boston, USA. 2MGH, Boston, USA. 3Boston Children's Hospital, Boston, USA

4. Exosome secreted by hSOD1G93A and prpTDP-43A315T cerebral cortex induce an early disease modulation signal

Mina Peric1,2, Edward Xie1, Nuran Kocak1, Mukesh Gautam1, Johnatan Brent1, Nicholas Angeloni3, Shad Thaxton3,4, Hande Ozdinler1,5,6,7
1Davee Department of Neurology and Clinical Neurological Sciences, Northwestern University, Chicago, USA. 2Center for Laser Microscopy, Institute of Physiology and Biochemistry, Faculty of Biology, University of Belgrade, Belgrade, Serbia. 3Department of Urology, Feinberg School of Medicine, Northwestern University, Chicago, USA. 4International Institute for Nanotechnology, Northwestern University, Evanston, USA. 5Robert H. Lurie Comprehensive Cancer Center, Chicago, USA. 6Cognitive Neurology and Alzheimer's disease Center, Chicago, USA. 7Les Turner ALS Center Northwestern University Feinberg School of Medicine, Chicago, USA
5. Mitochondria of upper motor neurons with TDP-43 pathology undergo mitoautophagy, a unique self-destructive path, very early in ALS

Mukesh Gautam1, Edward Xie1, Nuran Kocak1, Hande Ozdiner1,2,3,4
1Davee Department of Neurology and Clinical Neurological Sciences Northwestern University, Chicago, USA.
2Les Turner ALS Center, Northwestern University, Chicago, USA.
3Mesulam Cognitive Neurology and Alzheimer’s Disease Center, Northwestern University, Chicago, USA.
4Robert H. Lurie Comprehensive Cancer Research Center, Northwestern University Feinberg School of Medicine, Chicago, USA.

6. Focusing our attention from mice to neuron in drug discovery efforts and building better behavioral outcome measures for motor neuron circuitry in mice

Oge Gozutok1, Baris Genc1, Santana Sanchez1, Ina Dervishi1, Mukesh Gautam1, Richard Silverman2, P. Hande Ozdiner1,3
1Davee Department of Neurology and Clinical Neurological Sciences, Feinberg School of Medicine, Northwestern University, Chicago, IL, USA.
2Department of Chemistry, Northwestern University, Chicago, IL, USA.
3Les Turner ALS Center at Northwestern University, Chicago, IL, USA.

7. Assessment of novel compounds that improve corticospinal motor neuron health in vitro and in vivo

Baris Genc1, Oge Gozutok1, Ina Dervishi1, Santana Sanchez1, Nuran Kocak1, Hye Shin1, Edward Xie1, Mukesh Gautam1, Richard Silverman2, P. Hande Ozdiner1
1Davee Department of Neurology and Clinical Neurological Sciences, Northwestern University, Chicago, USA.
2Department of Chemistry, Northwestern University, Chicago, USA.

8. MCP1-CCR2 and neuroinflammation in the ALS motor cortex with TDP-43 pathology

NURAN KOCAK, Mukesh Gautam, Javier H Jara, Edward F Xie, Qinwen Mao, Eileen H Bigio, P. Hande Özdinler
NORTHWESTERN UNIVERSITY, CHICAGO, USA.

9. Nanocrystalline Gold as a Novel Biocatalytic Therapeutic for Amyotrophic Lateral Sclerosis

Joanne Zhang1, William Lee*2, Phillip Mortenson1, Lindsay Steinmetz1, David Pierce1, Mikhail Merzliakov1, Adam Dorfman1, Michael Hotchkin3, Karen Ho3, Mark Mortenson1
1Clene Nanomedicine, Inc., North East, USA.
2Co-1st Author, Clene Nanomedicine, Inc., North East, USA.
3Clene Nanomedicine, Inc., Salt Lake City, USA.

10. The cardiolipin-targeting compound SBT-272 attenuates neurodegeneration, delays the onset of neurological signs and extends lifespan in male SOD1 G93A transgenic mice

Dennis Keefe1, Guozhu Zheng2, Laurent Bogdanik3, Arie Mobley3, Inese Smutske1, Mark Bamberger1
1Stealth Biotherapeutics, Newton, MA, USA.
2Stealth Biotherapeutics, Newton, MA, USA.
3The Jackson Laboratory, Bar Harbor, ME, USA.
11. Protocatechuic acid significantly extends survival, improves motor function, and displays
neuroprotective and anti-inflammatory therapeutic benefits in the G93A mutant hSOD1 mouse
model of amyotrophic lateral sclerosis

Lilia Koza, Aimee Winter, Jessica Holsopple, Claudia Pena, Angela Aviso, Daniel Linseman
University of Denver, Denver, USA

12. Genetic pleiotropy causes novel central nervous system, skeletal muscle and cardiac pathology
that contribute to early death of transgenic mice expressing ALS-linked CHCHD10 p.R15L

Eanna Ryan, Hong Zhai, Erdong Liu, John Silva, Jing Su, Sudershan Dayanidhi, Yongchao Ma,
Han_Xiang Deng, Teepu Siddique
Northwestern University Feinberg School of Medicine, Chicago, USA

13. Blood-CSF barrier disruptions in ALS

Nadine Bakkar1, Justin Saul1, Elizabeth Hutchins2, Rebecca Reiman2, Sara Bowen1, Lyle Ostrow3, Brent Harris4,
Shafeeq Ladha1, Kendall Van-Keuren Jensen2, Robert Bowser1
1Barrow Neurological Institute, Phoenix, USA. 2Tgen, Phoenix, USA. 3Johns Hopkins University, Baltimore, USA.
4Georgetown University, Washington, USA

14. Glycolysis upregulation is neuroprotective as a compensatory mechanism in ALS

Ernesto Manzo1, Ileana Lorenzini2, Dianne Barrameda1, Jordan Barrows1, Alexander Starr2, Tina Kovalik2,
Benjamin Robichow2, Robert Bowser2, Rita Sattler2, Daniela Zarnescu1
1University of Arizona, Tucson, USA. 2Barrow Neurological Institute, Phoenix, USA

Disease Mechanism

15. Pathway analysis in ALS

Sara Saez-Atienza1, Sara Bandres-Ciga1, Jonggeol Kim1, Ruth Chia1, Michael Nalls1, Adriano Chio2, Bryan
Traynor1
1NIA, Bethesda, USA. 2University of Turin, Turin, Italy

16. Alleviating nucleocytoplasmic transport disruption in amyotrophic lateral sclerosis and
frontotemporal dementia by targeting FUS mislocalization

Nan Li1-2, Fernande Freyermuth1-2, Nibha Mishra1-2, Yi Han1, Corey Aguilar1, Samia Pratt1, Scott Mordecai3, Jin
Kim1, Patricia Rogers1, Michael Workman1-2, Ricardos Tabet1-2, Chao Lee1-2, Kitty Savage1-2, Melanie Jambeau1-2,
Philip Damme4, Kathryn Swoboda1, Roy Soberman5, James Berry1, Doo Kim1, Anne Bang6, Clotilde Lagier-
Tourenne1-2
1Department of Neurology, Sean M. Healey & AMG Center for ALS at Mass General, Massachusetts General
Hospital and Harvard Medical School, Boston, USA. 2Broad Institute of Harvard University and MIT, Cambridge,
17. Using extracellular vesicles to analyze ALS biomarkers in blood and urine

Laura Oakley, Maria Elena Cicardi, Kelly Cyliax, Christopher Hague, Katelyn Russell, Aaron Haeusler, Davide Trotti, Piera Pasinelli
Jefferson Weinberg ALS Center, Vickie and Jack Farber Institute for Neuroscience, Philadelphia, USA

18. Defining Early Markers of Disease in Familial ALS: An Interval Analysis of the DIALS Network Study

Katharine Nicholson, Isabel Anez-Bruzual, Katherine Burke, Diane Lucente, Maggie Clapp, Jennifer Jockel-Balsarotti, Amber Malcolm, Taylor Stirrat, Lindsay Pothier, Tania Gendron, Mercedes Prudencio, James Chan, Leonard Petrucelli, James Berry
1Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital, Boston, USA. 2Washington University, Saint Louis, USA. 3Mayo Clinic, Jacksonville, USA. 4Massachusetts General Hospital, Boston, USA


Alex M Dickens, Ammar Al-Chalabi, Pamela Shaw, P Nigel Leigh, Leonard van den Berg, Orla Hardiman, Albert Ludolph, Valtteri Aho, Toni Sarapohja, Elina Serkkola, Chris Garratt, Kira M Holmström
1Orion Pharma, Orion Corporation, Espoo, Finland. 2Department of Basic and Clinical Neuroscience, King’s College London, Maurice Wohl Clinical Neuroscience Institute, London, United Kingdom. 3Department of Neurology, King’s College Hospital, London, United Kingdom. 4Sheffield Institute for Translational Neuroscience and NIHR Sheffield Biomedical Research Centre, University of Sheffield, Sheffield, United Kingdom. 5Department of Neuroscience Brighton and Sussex Medical School, Trafford Centre for Biomedical Science, Falmer, Brighton, United Kingdom. 6Department of Neurology, University Medical Center Utrecht, Utrecht, Netherlands. 7Department of Neurology, University of Ulm, Ulm, Germany. 8Orion Pharma, Orion Corporation, Nottingham, United Kingdom

20. NEALS Biorepository: A “Living Library”

Dario Gelevski, Miriam Moscovitch-Lopatin, Alanna Farrar, Isabel Anez, Cassandra Lieberman, Lizzi Neylon, Tina Kovalik, Tara Lincoln, Terry Heiman-Patterson, Robert Bowser, James Berry
1Neurological Clinical Research Institute, Department of Neurology, Massachusetts General Hospital, Boston, USA. 2Barrow Neurological Institute at Dignity Health at St. Joseph’s Hospital and Medical Center, Phoenix, USA. 3Northeast Amyotrophic Lateral Sclerosis Consortium, Boston, USA. 4Center for Neurodegenerative Disorders, Temple Health, Philadelphia, USA

Eufrosina Young\textsuperscript{1,2}, David Mclain\textsuperscript{3}, Lauren Warren-Farcy\textsuperscript{1}, Claudine Ward\textsuperscript{4}, Susama Verma\textsuperscript{2}, Steven Brose\textsuperscript{2}, Servatius Richard\textsuperscript{2}

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Bulbar

22. Validation of Yale Swallow Protocol in ALS: Preliminary Results

Kendrea Garand\textsuperscript{1}, Debra Suiter\textsuperscript{2}, Stephanie Reyes\textsuperscript{3}, Justine Allen\textsuperscript{4}, Michelle Moore\textsuperscript{5}, Amy Chen\textsuperscript{6}

\textsuperscript{1}University of South Alabama, Mobile, USA. \textsuperscript{2}University of Kentucky, Lexington, USA. \textsuperscript{3}Augusta University, Augusta, USA. \textsuperscript{4}University of Florida, Gainsville, USA. \textsuperscript{5}Medical University of South Carolina, Charleston, USA

23. Cough strength is associated with maximal inspiratory and expiratory pressure capacity, inspiratory cough flow, and forced vital capacity in ALS.

Kasey McElheny\textsuperscript{1}, Jennifer Chapin\textsuperscript{1}, Lauren DiBiase\textsuperscript{1}, Amber Anderson\textsuperscript{1}, Lauren Tabor\textsuperscript{2}, James Wymer\textsuperscript{1}, Emily Plowman\textsuperscript{1}

\textsuperscript{1}University of Florida, Gainesville, USA. \textsuperscript{2}Phil Smith Neuroscience Institute at Holy Cross Hospital, Fort Lauderdale, USA

24. Use of the Beiwe Smartphone App to Identify and Track Bulbar Impairment in ALS

Kathryn Connaghan\textsuperscript{1}, Harli Weber\textsuperscript{2}, Jordan Green\textsuperscript{1}, Sabrina Paganoni\textsuperscript{3,2}, James Chan\textsuperscript{4}, Ella Collins\textsuperscript{2}, Brian Richburg\textsuperscript{1}, Marziye Eshghi\textsuperscript{1}, JP Onnela\textsuperscript{5}, James Berry\textsuperscript{3,2}

\textsuperscript{1}MGH Institute of Health Professions, Boston, USA. \textsuperscript{2}Neurological Clinical Research Institute, Department of Neurology, Massachusetts General Hospital, Boston, USA. \textsuperscript{3}Harvard Medical School, School of Medicine, Boston, USA. \textsuperscript{4}Massachusetts General Hospital Department of Biostatistics, Boston, USA. \textsuperscript{5}T.H. Chan Harvard School of Public Health, Boston, USA

25. Profiles of Dysarthria and Dysphagia in ALS

Lauren DiBiase, Amber Anderson, Justine Allen, Jennifer Chapin, Amy Ashley, Kasey McElheny, Julia Eckart, Kelly Leonard, Rael Robison, May Smith-Sherry, Kelby Magennis, James Wymer, Emily Plowman

University of Florida, Gainesville, USA

26. Using voice analysis to track ALS progress in clinical trials with a mobile app

Shira Hahn\textsuperscript{1}, Gabriela Stegmann\textsuperscript{1}, Visar Berisha\textsuperscript{1,2}, Julie Liss\textsuperscript{1,2}, Bettina Cockcroft\textsuperscript{3}, Fady Malik\textsuperscript{3}, Lisa Meng\textsuperscript{3}, Stacy Rudnicki\textsuperscript{3}, Andrew Wolff\textsuperscript{3}, Jeremy Shefrin\textsuperscript{4}

\textsuperscript{1}Aural Analytics, Scottsdale, USA. \textsuperscript{2}Arizona State University, Tempe, USA. \textsuperscript{3}Cytokinetics, South San Francisco, USA. \textsuperscript{4}Barrow Neurological Institute, Phoenix, USA
27. Impedance pharyngography to evaluate swallowing in patients with amyotrophic lateral sclerosis

Fu Zhang, Hilda Gutierrez, Akashleena Mallick, Badria Munir, Sarah MacKenzie, Hawa Yusuf, Seward Rutkove
Beth Israel Deaconess, Boston, USA

28. Feasibility and Implementation of a Bulbar Data Collection Tool in a Multidisciplinary ALS Clinic

Lauren Tabor Gray¹, Fiona Scarlett¹, Gabriela Lopes¹, Joelle Simpson², Eduardo Locatelli¹
¹Phil Smith Neuroscience Institute, Holy Cross Hospital, Fort Lauderdale, USA. ²University of Florida, Gainesville, USA

29. Development of the ALS Index of Bulbar Dysfunction (ALS-IBD): Face and Content Validity

Ashley A Waito¹,², Jordan R Green³, Carolina Barnett Tapia²,⁴, Rosemary Martino²,⁴, Agessandro Abrahao¹, Lorne Zinman¹,², Yana Yunusova²,¹,⁴
¹Sunnybrook Research Institute, Toronto, Canada. ²University of Toronto, Toronto, Canada. ³Massachusetts General Hospital, Boston, USA. ⁴University Health Network, Toronto, Canada

30. Longitudinal Change of Speech Timing Measures in Individuals with ALS

Ashley Waito¹,², Chelsea Tanchip², Cindy Cui¹, Reeman Marzouqah¹,², Carolina Barnett Tapia²,³, Agessandro Abrahao¹, Lorne Zinman¹,², Jordan R Green⁴, Yana Yunusova²,¹,³
¹Sunnybrook Research Institute, Toronto, Canada. ²University of Toronto, Toronto, Canada. ³University Health Network, Toronto, Canada. ⁴Massachusetts General Hospital, Boston, USA

Clinical Care

31. TIGLUTIK™ (Riluzole Oral Suspension ITF2985) in a Percutaneous Endoscopic Gastrostomy (PEG)-Modeled Bioequivalence Study: A Randomized, 2-way Crossover, Pharmacokinetic (PK) Comparison of Intragastric with Oral Administration

Benjamin Rix Brooks, MD¹,²,³, Paolo Bettica, MD, PhD⁴, Sara Cazzaniga⁴
¹Carolinas Medical Center, Charlotte, USA. ²Atrium Health, Charlotte, USA. ³University of North Carolina School of Medicine, Charlotte, USA. ⁴Italfarmaco S.p.A, Milan, Italy

32. Wearable gait sensors for continuous estimation of fall risk in ALS

Andrew Geronimo, Zachary Simmons
Penn State College of Medicine, Hershey, USA

33. Utilization of telehealth for ALS care

Anne Haulman, Amit Chahwala, Andrew Geronimo, Zachary Simmons
Penn State Health Hershey Medical Center, Hershey, USA
34. Growing hope: Incorporation of social media increases attendance at in-person resource/support groups

Alair Altiero\textsuperscript{1,2}, Anne Haulman\textsuperscript{2}, Susan Walsh\textsuperscript{1,2}, Zachary Simmons\textsuperscript{2}
\textsuperscript{1}ALS Association Greater Philadelphia Chapter, Harrisburg, USA. \textsuperscript{2}Penn State Health Hershey Medical Center, Hershey, USA

35. Demographics, Needs, and Educational Interests of Caregivers of Individuals with ALS

Jerrica Farias, Niraja Suresh, Samuel Dang, Natalie Tucker, Brittany Harvey, Clifton Gooch, Lucy Lam, Allison Schleutker, Erik Velasquez, Brittney Mullins, Tuan Vu
University of South Florida, Tampa, USA

36. Nursing Driven Initiative To Increase Tolerability and Compliance of BHV0223 Novel Therapy in Patients Diagnosed with ALS

Lisa Ranzinger, MSN, RN, Allison Newell-Sturdivant, BSN, RN, CCRC, Johnny Jones, MS, Benjamin Brooks, MD
Atrium Health, Carolinas Neuromuscular/ALS-MDA Care Center, Charlotte, USA

37. Impact of dysphagia and gastrostomy on quality of life in caregivers of patients with ALS

Emily Goggin, Debra Suiter, Edward Kasarskis, Meha Joshi
University of Kentucky, Lexington, USA

38. Reduced Lingual Strength is Related to Increased effort and Decreased Efficiency of Swallowing Individuals with ALS

Raele Robison, Jennifer Chapin, Lauren DiBiase, Amber Anderson, Kelby Magennis, James Wymer, Emily Plowman
University of Florida, Gainesville, USA

39. Predictors of telehealth utilization for ALS care

Anne Haulman, Andrew Geronimo, Zachary Simmons
Penn State Health Hershey Medical Center, Hershey, USA

40. Comparing the efficacy of stretching brochures to videos for increasing adherence to stretching exercises in individuals with motor neuron disease.

Katherine Burke\textsuperscript{1,2}, Fabiola De Marchi\textsuperscript{3}, Amy Ellrodt\textsuperscript{1}, Michael Doyle\textsuperscript{1}, Megha Kouli\textsuperscript{3}, Olivia Comeau\textsuperscript{1}, Elizabeth Adelson\textsuperscript{1}, Rebecca Walter\textsuperscript{2}, Melissa Kusy\textsuperscript{2}, Flor Amaya\textsuperscript{2}, Carissa Anderson\textsuperscript{2}, Jennifer Honda\textsuperscript{2}, James Chan\textsuperscript{3}, James Berry\textsuperscript{1}, Sabrina Paganoni\textsuperscript{1,4,5}
\textsuperscript{1}Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, Department of Neurology, Harvard Medical School, Boston, USA. \textsuperscript{2}Massachusetts General Hospital, Institute of Health Professions (IHP), Boston, USA. \textsuperscript{3}Department of Biostatistics, Massachusetts General Hospital, Harvard Medical School, Boston, USA.
41. Does Pseudobulbar Affect influence Healthcare Conversations in ALS patients?
Aditi Varma-Doyle, Nicole Villemarette-Pittman, Brian Copeland
LSUHSC, New Orleans, USA

42. Prevalence of ALS-FTSD at VCU and Development of a New Diagnostic and Clinical Care Algorithm for Patients and Families
Kelly Gwathmey, Kiera Berggren
Virginia Commonwealth University, Richmond, USA

43. Identifying and Assessing Preferences Regarding Advance Care Planning, Psychosocial Needs, and End of Life Wishes among ALS patients
Lalanthica V. Yogendran MD. MPH., Bianca Barcelo MD., Dominique Mortel MD., Bradley Russell Shane, Briana-Linnette Ibarra, Catherine Bree Johnston MD. MPH., Holli Horak MD., Katalin Scherer MD.
University of Arizona, Tucson, USA

44. The ALS Genetic Access (GAP) Program: Paving the Way for Genetic Characterization of ALS in the Clinic
Jennifer Roggenbuck¹, Leah Vicini¹, Carly Doyle², Tara Lincoln², Jonathan Glass³
¹The Ohio State University Wexner Medical Center, Columbus, USA. ²The Northeast ALS Consortium, Los Angeles, USA. ³Emory University School of Medicine, Atlanta, USA

45. AM-PAC as a Measure of functional independence in patients with ALS
Angelica Gicalone, Gleydiane De Oliveira, Shah Jaimin, Bjorn Oskarsson
Mayo Clinic, Jacksonville, USA

46. Real-World Evidence of Radicava® (edaravone) for Amyotrophic Lateral Sclerosis From a National Infusion Center Database in the United States
Terry Heiman-Patterson¹, Johnna Perdrizet², Stephen Apple², Barbara Prosser³, Wendy Agnese²
¹Temple University Lewis Katz School of Medicine, Philadelphia, USA. ²Mitsubishi Tanabe Pharma America, Inc., Jersey, USA. ³Soleo Health, Sharon Hill, USA

47. Laryngectomy in ALS at Mayo Clinic Jacksonville, 2009 to 2019
Bjorn Oskarsson¹, Jaimin Shah², Phillip Pirgousis², Sarah Reising², Janay Caradonna², Jany Paulette², William Freeman²
¹Mayo Clinic, Jacksonville, USA. ²
48. One Academic Center's Experience with Edaravone

Paula Brockenbrough, Rebecca Rhodes, Kathleen Pearson, Scott Vota, Kelly Gwathmey
VCU Health, Richmond, USA

49. A Preliminary Analysis of the Feasibility and Efficacy of Edavarone at a Multidisciplinary ALS Clinic

Lauren Tabor Gray, Fiona Scarlett, Maricela Pereda, Gabriela Lopes, Eduardo Locatelli
Phil Smith Neuroscience Institute, Holy Cross Hospital, Fort Lauderdale, USA

50. Leap2BFit Supplementation in Individuals with ALS

Lauren Tabor Gray, Fiona Scarlett, Gabriela Lopes, Gustavo Alameda, Eduardo Locatelli
Phil Smith Neuroscience Institute, Holy Cross Hospital, Fort Lauderdale, USA

51. Early Treatment Effects of Riluzole in ALS-MND 2. Isometric Strength Improvements in Sentinel Muscles

Benjamin Rix Brooks\textsuperscript{1,2}, Elena K Bravver\textsuperscript{1,2}, Urvi G Desai\textsuperscript{1,2}, Navid Jalali\textsuperscript{1,2}, William L Bockenek\textsuperscript{1,2}, Scott S Lindblom\textsuperscript{1,2}
\textsuperscript{1}Carolinias Neuromuscular / ALS MDA Care Center, Charlotte, USA. \textsuperscript{2}University of North Carolina School of Medicine - Charlotte Campus, Charlotte, USA

52. Early Treatment Effects of Riluzole in ALS-MND 1. Correction of Hand Grip Apraxia in ALS-FTD

Benjamin Rix Brooks\textsuperscript{1,2}, Elena K Bravver\textsuperscript{1,2}, Urvi G Desai\textsuperscript{1,2}, Navid Jalali\textsuperscript{1,2}, William L Bockenek\textsuperscript{1,2}, Scott S Lindblom\textsuperscript{1,2}
\textsuperscript{1}Carolinias Neuromuscular / ALS MDA Care Center, Charlotte, USA. \textsuperscript{2}University of North Carolina School of Medicine - Charlotte Campus, Charlotte, USA

53. The Effect of Feeding Tube Placement on Body Mass Index and Amyotrophic Lateral Sclerosis Functional Rating Scale Revised

Rebecca Rhodes, Paula Brockenbrough, Scott Vota, Kelly Gwathmey
Virginia Commonwealth University Health System, Richmond, USA

54. Will improving patient and caregiver care experience for ALS patients improve participation in research trials?

Chelsey Carter, Grace Gerbi
Washington University in St. Louis, St. Louis, USA
Pulmonary

55. Non-invasive Negative Pressure Ventilation in a patient with advanced ALS

Sam Maiser1, Steven Lufkin2
1Hennepin Healthcare, Minneapolis, USA. 2ALS Patient at Hennepin Healthcare, Minneapolis, USA

56. Improving use of non-invasive ventilation using custom 3D-printed mask cushion interfaces for persons with ALS

Stephen Goutman, Jeffrey Plott, Lei Chen, Kyle VanKoevering, Albert Shih, Glenn Green
University of Michigan, Ann Arbor, USA

57. Agree to Disagree: Clinician Practice Patterns for ALS Respiratory Care

Jason Ackrivo1, John Hansen-Flaschen1, Lauren Elman1, Terry Heiman-Patterson2, Steven Kawut1
1University of Pennsylvania, Philadelphia, USA. 2Temple University, Philadelphia, USA

58. The Earlier the Better? How ALS Patients Feel about Respiratory Care

Jason Ackrivo1, John Hansen-Flaschen1, Lauren Elman1, Terry Heiman-Patterson2, Steven Kawut1
1University of Pennsylvania, Philadelphia, USA. 2Temple University, Philadelphia, USA

59. ALS Patients Performing Overnight Oximetry in Their Home, a Process Improvement Project.

Bradley Boynton, Nathan Staff, Karla Folkerts, Darcy McGowan, Todd Meyer
Mayo Clinic, Rochester, MN, USA

60. Electrical impedance tomography for the assessment of pulmonary function in ALS patients

Ethan Murphy1, Fu Zhang2, Badria Munir2, Akashleena Mallick2, Hilda Gutierrez2, Christy Smith2, Sean Levy2, Courtney McIlduff2, Ryan Halter1, Seward Rutkove2
1Dartmouth College, Hanover, USA. 2Beth Israel Deaconess Medical Center, Boston, USA

Clinical Trials

61. Baseline characteristics and status update of REFALS: a phase 3 study comparing oral levosimendan to placebo in patients with ALS

Merit Cudkowicz1, Angela Genge2, Nicholas Maragakis3, Susanne Petri4, Leonard van den Berg5, Valtteri Aho6, Chris Garratt6, Toni Sarapohja6, Ammar Al-Chalabi7
1Massachusetts General Hospital, Boston, USA. 2Montreal Neurological Institute and Hospital, Montreal, Canada. 3Johns Hopkins University, Baltimore, USA. 4Medizinische Hochschule Hannover, Hannover, Germany. 5University Medical Center Utrecht, Utrecht, Netherlands. 6Orion Pharma, Orion Corporation, Espoo, Finland. 7King’s College London, London, United Kingdom
62. ALS AT HOME: Novel approaches to recruiting, enrollment, and retention in a remote study

Kerisa Shelton1, Seward Rutkove2, Jeremy Shefner1
1Barrow Neurological Institute, Phoenix, USA. 2Beth Israel Deaconess Medical Center, Boston, USA

63. Triheptanoin Is Poorly Tolerated and Does Not Significantly Slow Progression, Improve MR Spectroscopy, or Influence Selected Biomarkers in a Small Pilot Trial of People with ALS

Richard Bedlack, Cecil Charles, Ivan Spasojevic, Michael Lutz
Duke University, Durham, USA

64. The Frazier Free Water Protocol: A case study intervention for dysphagia and aspiration pneumonia

Elizabeth Kelley1, Michelle McDonagh1, Dominic Fee2, Paul Barkhaus2
1Froedtert Hospital, Milwaukee, USA. 2Medical College of Wisconsin, Milwaukee, USA

65. The REFALS-ES open-label extension study of oral levosimendan (ODM-109)

Merit Cudkowicz1, Angela Genge2, Nicholas Maragakis3, Susanne Petri4, Leonard van den Berg5, Valtteri Aho6, Chris Garratt6, Toni Sarapohja6, Ammar Al-Chalabi7
1Massachusetts General Hospital, Boston, USA. 2Montreal Neurological Institute and Hospital, Montreal, Canada. 3Johns Hopkins University, Baltimore, USA. 4Medizinische Hochschule Hannover, Hannover, Germany. 5University Medical Center Utrecht, Utrecht, Netherlands. 6Orion Pharma, Orion Corporation, Espoo, Finland. 7King’s College London, London, United Kingdom

66. The Rasch-Built Overall ALS Disability Scale: ROADS to a better ALS outcome measure

Christina Fournier1,2, Richard Bedlack3, Colin Quinn4, James Russell5, Diane Beckwith2, Kathleen Kaminski1, William Tyor1,2, Vicki Hertzberg2, Virginia James2, Meraida Polak2, Jonathan Glass2
1Atlanta VA Medical Center, Atlanta, USA. 2Emory University, Atlanta, USA. 3Duke University, Durham, USA. 4University or Pennsylvania, Philadelphia, USA. 5Lahey Clinic, Burlington, USA

67. Creation of the ALS Nutrition App for the E-health Application To Modify ORal Energy intake and Measure Outcomes REmotely in ALS Clinical Trial (EAT MORE2)

Mansi Sharma, Jane Hubbard, James Chan, James Berry, Anne-Marie Wills
Massachusetts General Hospital, Boston, USA

68. Interim analysis of first in human clinical trial using human astrocytes (AstroRx®) for the treatment of ALS

Marc Gotkine1, Yosef Lerner1, Yael Feinsod-Meiri2, Michal Izrael3, Tamir Ben-Hur1, Judith Chebath3, Arik Hasson3, Guy Slutsky3, Yosef Caraco2, Michel Revel3,4
69. Design of a Phase 3, Randomised, Placebo-Controlled Trial of oral Arimoclomol in Amyotrophic Lateral Sclerosis (ORARIALS-01)

Claus Sundgreen, Thomas Blaettler, Richard Bennett, Dror Rom, Peter M Andersen, Joanne Wuu, Michael Benatar
Orphazyme A/S, Copenhagen, Denmark. Prosoft Clinical Inc, Huntingdon Valley, USA. Department of Pharmacology and Clinical Neuroscience, Umeå University, Umeå, Sweden. Department of Neurology, University of Miami, Miami, USA

70. Open Label Clinical Trial of MN-166 (Ibudilast) in Amyotrophic Lateral Sclerosis (ALS) – A biomarker endpoint-based clinical trial.

Suma Babu, Baileigh Hightower, Nicole Zurcher, Chieh-En Tseng, Catherine Cebulla, Danica Sanders, Olivia Pijanowski, Haruhiko Banno, Joanna Dojillo, James Chan, Kazuko Matsuda, Mark Gudesblatt, Merit Cudkowicz, Jacob Hooker, Nazem Atassi
Sean M Healey & AMG Center for ALS, Massachusetts General Hospital, Boston, USA. A. A. Martinos Center for Biomedical Imaging, Department of Radiology, Massachusetts General Hospital, Boston, USA. Medicinova Inc, La Jolla, USA. Department of Biostatistics, Massachusetts General Hospital, Boston, USA. South Shore Neurologic Associates, Patchogue, USA

71. Bioimpedance Data as Potential Markers of Clinical Course in Amyotrophic Lateral Sclerosis: An Ongoing Longitudinal Study to Predict Functionality

William Mays, Tulio Bertorini, Jeffrey Metter, Laura Talbot, Khadija Awais, Andrei Alexandrov
UTHSC, Memphis, USA

72. Detectable Effect Cluster Analysis: A Novel Machine-Learning Based Clinical Trial Subgroup Analysis Tool

Danielle Beaulieu*, Albert Taylor*, Andrew Conklin, Jonavelle Cuerdo, Dustin Pierce, Mike Keymer, David Ennist
Origent Data Sciences, Inc., Washington, DC, USA

73. CSF MCP-1: A surrogate biomarker for ALS

Ralph Kern, James Berry, Revital Aricha, Haggai Kaspi, Merit Cudkowicz, Anthony Windebank, Nathan Staff, Margaret Ayo Owegi, Yossef S. Levy, Chaim Lebovits, Robert Brown, Yael Gothelf
Brainstorm Cell Therapeutics, Petach Tikva, Israel. Massachusetts General Hospital, Boston, USA. Mayo Clinic, Rochester, USA. UMass Medical School, Worcester, USA
74. Lung volume recruitment combined with expiratory muscle strength training to improve ventilatory, cough, swallow, and speech function in ALS

David Walk¹, Emily Plowman², Wymer James², Peter Watson¹, Michael Shyne¹, Valerie Ferment¹, Kelby Magennis², Carol Smith², Megan Somers¹, Jennifer Chapin², Nancy Nasi¹, Karen Kosieracki¹, Lauren DiBiase², Amber Anderson²
¹University of Minnesota, Minneapolis, USA. ²University of Florida, Gainesville, USA

75. Engaging ALS Research Ambassadors to Help Design the REFINE-ALS Biomarker Study

James Berry¹, Richard Bedlack², Debra Mathews³, Wendy Agnese⁴, Stephen Apple⁴
¹Massachusetts General Hospital, Boston, USA. ²Duke University School of Medicine, Durham, USA. ³Johns Hopkins Berman Institute of Bioethics, Baltimore, USA. ⁴Mitsubishi Tanabe Pharma America, Inc., Jersey City, USA

76. Evidence for Generalizability of Edaravone Efficacy Using a Novel Machine-Learning (ML) Risk-Based Analysis Tool

Benjamin Brooks¹, Erik Pioro², Mark Schactman³, Danielle Beaulieu⁴, Albert Taylor⁴, Mike Keymer⁴, Wendy Agnese⁵, Johnna Perdrizet⁵, Stephen Apple⁵, David Ennist⁴
¹Carolina Neuromuscular/ALS-MDA Center in Charlotte, Charlotte, USA. ²Section of ALS & Related Disorders, Cleveland Clinic, Cleveland, USA. ³Firma Clinical Research, Hunt Valley, USA. ⁴Origent Data Sciences, Vienna, USA. ⁵Mitsubishi Pharma America, Inc. (MTPA), Jersey City, USA

77. FORTITUDE-ALS: Who received the most benefit, and translation to real world events

Jeremy M. Shefner¹, Jinsy A. Andrews², Angela Genge³, Carlayne Jackson⁴, Noah Lechtzin⁵, Timothy M. Miller⁶, Bettina M. Cockroft⁷, Fady I. Malik⁷, Lisa Meng⁷, Jenny Wei⁷, Andrew A. Wolff⁷, Stacy A. Rudnicki⁷
¹Barrow Neurological Institute, Phoenix, USA. ²Columbia University, New York, USA. ³Montreal Neurological Institute and Hospital, Montreal, Canada. ⁴University of Texas Health Science Center, San Antonio, USA. ⁵Johns Hopkins School of Medicine, Baltimore, USA. ⁶Washington University School of Medicine, St. Louis, USA. ⁷Cytokinetics, Inc., South San Francisco, USA

78. Interleukin 6 Receptor Asp358Ala Variant May influence Effectiveness of IL6 Blocking Therapies

Phonepasong Arounleut¹, Nazem Atassi, MD², Samu Babu, MD³, Richard Barohn, MD⁴, Robert Bowser, PhD⁵, James Caress, MD¹, Armineuza Evora³, Gregory Hawkins, PhD¹, Shafeeq Ladha, MD², Tina Kovalik⁵, Eric Macklin, PhD³, Carol Milligan, PhD¹, Jeremy Shefner, MD, PhD⁶, Zachary Simmons, MD⁶, Alexander Starr⁵, Marlena Wosiski-Kuhn¹
¹Wake Forest School of Medicine, Winston-Salem, USA. ²Sanofi, Cambridge, USA. ³Massachusetts General Hospital, Boston, USA. ⁴University of Kansas Medical Center, Fairway, USA. ⁵Barrow Neurological Institute, Phoenix, USA. ⁶Penn State Hershey Medical Center, Hershey, USA
79. Retrospective chart review of carbidopa-levodopa for treatment of spasticity

William Everett, Yu-Ting Chen, Hillary Herzog, Amber Malcolm, Jennifer Jockel-Balsarotti, Timothy Miller
Washington University, St. Louis, USA

80. Repurposing anticancer drugs for the treatment of ALS.

Thomas Lukas, Teepu Siddique
Northwestern University Feinberg School of Medicine, Chicago, USA

81. Using Smartphone Data as a Digital Phenotyping Platform to Quantify ALS Progression

Ella Collins¹, Harli Weber¹, Katherine Burke¹, Kenzie Carlson², Joel Salinas³, James Chan⁴, Josh Barback², Kathryn Conaghan⁵, Jordan Green⁵, Jukka-Pekka Onnela², Sabrina Paganoni⁶,⁷, James Berry¹
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82. NeuroREACH™ Platform for Extended Access Program (EAP) Trials as the Foundation for Clinical Research of the Future

Alexander Sherman¹,², Amanda Podesta¹, Kenneth Faulconer¹, Natalia Tarasenko¹, Jason Walker¹, Hong Yu¹, Derek D’Agostino¹, Merit Cudkowicz¹,²
¹MGH, Boston, USA. ²Harvard Medical School, Boston, USA

Epidemiology

83. Risk of ALS in Italian professional soccer leagues, an epidemiological cohort-study

Elisabetta Pupillo¹, Elisa Bianchi¹, Nicola Vanacore², Carla Montalto¹, Giuseppe Ricca¹, Francesco Saverio Robustelli della Cunà³, Fabio Fumagalli⁴, Ettore Beghi¹
¹Mario Negri Institute for Pharmacological Research IRCCS, Milan, Italy. ²Istituto Superiore di Sanità, Roma, Italy. ³Università degli studi di Pavia, Pavia, Italy. ⁴Università degli studi di Milano, Milan, Italy

84. Conugal Amyotrophic lateral sclerosis: coincidence or environmental factors? A report on two couples and review of the literature.

Cuiping Zhao¹,², Miguel Chuquilin², James Wymer²
¹Department of Neurology, Qilu Hospital, Shandong University,, Jinan, China. ²McKnight Brain Institute, Department of Neurology, University of Florida College of Medicine, Gainesville, USA
85. Physical Activity and early onset amyotrophic lateral sclerosis (ALS), Data from the National ALS Registry: 2010 – 2018

Paul Mehta MD, Jaime Raymond MPH, Theodore Larson MS, Kevin Horton DrPH
National ALS Registry, CDC/ATSDR, Atlanta, USA

86. Unique metabolomic signatures in ALS participants based on persistent organic pollutant plasma concentrations

Stephen Goutman¹, Jonathan Boss¹, Sehee Kim¹, Kai Guo², Junguk Hur², Bhramar Mukherjee¹, Stuart Batterman¹, Eva Feldman¹
¹University of Michigan, Ann Arbor, USA. ²University of North Dakota, Grand Forks, USA

87. Pre-diagnostic cholesterol levels and the risk of amyotrophic lateral sclerosis

Kjetil Bjornevik¹, Éilis J. O’Reilly¹,², Laurence N. Kolonel³, Loic Le Marchand³, Marjorie L. McCullough⁴, Sabrina Paganoni⁵,⁶, Michael A. Schwarzschild⁵,⁶, Aladdyn H. Shadyab⁷, JoAnn E. Manson⁸,¹, Alberto Ascherio¹,⁸
¹Harvard T.H. Chan School of Public Health, Boston, USA. ²University College Cork, Cork, Ireland. ³University of Hawaii Cancer Center, Honolulu, USA. ⁴American Cancer Society, Atlanta, USA. ⁵Massachusetts General Hospital, Boston, USA. ⁶Harvard Medical School, Boston, USA. ⁷University of California, San Diego, USA. ⁸Brigham and Women’s Hospital, Harvard Medical School, Boston, USA

88. Determining Environmental Risk Factors for ALS using Large Claims and Environmental Pollutant Databases

Christopher Miller¹, Theresa Arndt¹, Pierantonio Russo¹, Oodaye Shukla¹, Charlotte Merrill², Wendy Agnese², Antoinette Harrison², Stephen Apple², Walter Bradley³, Elijah Stommel⁴, Angeline Andrew⁴, Xun Shi⁴, Tanya Butt⁴, Bart Guetti⁴
¹HVH Precision Analytics LLC, Wayne, USA. ²Mitsubishi Tanabe Pharma America, Inc. (MTPA), Jersey City, USA. ³Department of Neurology, Miller School of Medicine, University of Miami, Miami, USA. ⁴Dartmouth-Hitchcock Medical Center, Lebanon, USA

89. Amyotrophic Lateral Sclerosis and Multiple Sclerosis: More Evidence Suggesting a Link

Michael Elliott, Idil Baysal, Peiqing Qian, Angeli Mayadev, Jennifer Cardey, James Scanlan
Swedish Neuroscience Institute, Seattle, USA

90. Identification and Recruitment of Controls for the National ALS Registry Cases

Angela Malek¹, Todd Bear², Judith Rager², Abigail Foulds², Sarah DePerrior², Paul Mehta³, Jaime Raymond⁴, Kevin Horton³, Laurie Wagner⁴, Wendy Kaye⁴, John Vena¹, Evelyn Talbott²
¹Medical University of South Carolina, Charleston, USA. ²University of Pittsburgh, Pittsburgh, USA. ³Agency for Toxic Substances and Disease Registry (ATSDR)/Centers for Disease Control and Prevention (CDC), Atlanta, USA. ⁴McKing Consulting Corporation, Atlanta, USA
92. A Proposed Retrospective Research on Increased Risk of ALS and Neurodegeneration Among American Football Players

Isaac Whitworth\textsuperscript{1}, Alex Sherman\textsuperscript{1}, Jason Walker\textsuperscript{2}, Ervin Sinani\textsuperscript{1}, Amanda Nichols\textsuperscript{1}
\textsuperscript{1}MGH/NCRI, Boston, USA. \textsuperscript{2}MGH/NCRI, Boston, USA

Imaging

93. Measuring Upper Motor Neuron Dysfunction in Patients with ALS Using Transcranial Magnetic Stimulation

Armin Maghsoudlou, Adel Marei, Robin Warner, Mona Shahbazi, Shara Holzberg, Dale Lange
Hospital for Special Surgery, New York, USA

94. Magnetic Resonance Cytography based quantification of muscle degeneration in Amyotrophic Lateral Sclerosis

Sudarshan Ragunathan, Laura Bell, Ashley Stokes, Natanael Semmineh, Nicole Turcotte, Kerisa Shelton, Jessie Duncan, Shafeeq Ladha, Chad Quarles
Barrow Neurological Institute, Phoenix, USA

95. Observing patterns in MRI with QSM in Patients with C9ORF72 Familial ALS

Robin Warner\textsuperscript{1}, Apostolos Tsouris\textsuperscript{2}, Andrew D. Schweitzer\textsuperscript{2}, Mona Shahbazi\textsuperscript{1}, Dale Lange\textsuperscript{1}
\textsuperscript{1}Hospital for Special Surgery, New York, USA. \textsuperscript{2}Weill Cornell Medical Center, New York, USA

Database

96. The use and frequency of cannabinoid among patients with ALS

Erica Doon, Radwa Aly, Lindsey Covington, Elham Bayat
GWU, Washington, USA

97. Multivariate analysis of survival in an amyotrophic lateral sclerosis clinic population

Jaimin Shah\textsuperscript{1}, Kevin Boylan\textsuperscript{1}, Marka Van Blitterswijk\textsuperscript{1}, Rosa Rademakers\textsuperscript{1,2}, Otto Pedraza\textsuperscript{1}, Beth Rush\textsuperscript{1}, Jany Paulett\textsuperscript{1}, Janay Caradonna\textsuperscript{1}, Leonard Petrucelli\textsuperscript{2}, Bjorn Oskarsson\textsuperscript{1}
\textsuperscript{1}Mayo Clinic, Jacksonville, FL, USA. \textsuperscript{2}University of Antwerp, Antwerpen, Belgium

98. Combining culture-specific data display with culture-invariant data storage in PharmaENGINE™ improves international collaboration

Igor Katsovskiy, Alexander Sherman
MGH, Boston, USA
99. Clinical Research Support Optimization with the Universal Login Page

Yusra Wahab, Alexander Sherman, Alexander Korin
Mass General Hospital, Boston, USA

100. Automation of Data Transfer from EHR to EDC

Kenneth Faulconer¹, Alex Korin¹, Anne Vallis², Ximena Arcila-Londono², Tamela Stuchiner³
¹Massachusetts General Hospital, Boston, USA. ²Henry Ford Health System, Detroit, USA. ³Providence Brain and Spine Institute, Providence, USA

Other

101. Development of Patient Family Research Advisory Group

Susan Walsh¹,², Andrew Geronimo¹
¹Penn State Health, Hershey, USA. ²ALS Association Greater Philadelphia, Harrisburg, USA

102. ALS Clinical Research Learning Institutes (ALS-CRLI): Empowering People with ALS to be Research Ambassadors

Richard Bedlack¹, Allison Pogemiller², Jeremy Shefner³, Merit Cudkowicz⁴, Terri Heiman Patterson⁵
¹Duke University, Durham NC, USA. ²ALS Connect, Cave Creek AZ, USA. ³Barrow Neurological Institute, Phoenix AZ, USA. ⁴Mass General Hospital, Boston MA, USA. ⁵Temple University, Philadelphia PA, USA

103. The 'split elbow' and other observations from hand-held dynamometry.

Nimish Thakore², Brian Drawert², Brittany Lapin¹, Erik Pioro¹
¹Cleveland Clinic, Cleveland, OH, USA. ²University of North Carolina at Asheville, Asheville, NC, USA

104. Verb-Image Pair Test ( VIP )

Eufrosina Young¹,², David McLain³, Bei Yu⁴, Jun Wang⁴
¹SUNY Upstate, Syracuse, NY, USA. ²VA, Syracuse, NY, USA. ³SUNY Oswego, Oswego, NY, USA. ⁴Syracuse University, Syracuse, NY, USA

105. The Return of Answer ALS Results Study (RoAR): Answering the Duty to Disclose

Jennifer Roggenbuck¹, Amy Bartlett¹, Sarah Heintzman¹, Rory Eustace¹, Ashley Fox¹, Amy Knapp¹, Matthew Harms², Stephen Kolb¹
¹The Ohio State Wexner Medical Center, Columbus, USA. ²Columbia University, New York, USA

106. New NeuroGUID - NeuroSTAmP Generator User Management Service

Olga kharakozova¹, Igor Katsovskiy¹, Alexander Sherman²
107. Long-term survival in amyotrophic lateral sclerosis within the VA Biorepository Brain Bank

Thor Stein\textsuperscript{1,2}, Keith Spencer\textsuperscript{1}, Zachariah Foster\textsuperscript{1}, Nazifa Rauf\textsuperscript{1}, Derek Collins\textsuperscript{1}, James Averill\textsuperscript{3}, Sean Walker\textsuperscript{3}, Ian Robey\textsuperscript{3}, Neil Kowall\textsuperscript{1}, Christopher Brady\textsuperscript{1}
\textsuperscript{1}VA Boston Healthcare System, Boston, USA. \textsuperscript{2}Boston University School of Medicine, Boston, USA. \textsuperscript{3}Southern Arizona VA Healthcare System, Tucson, USA

108. Understanding & Addressing Barriers to ALS Clinical Trial Enrollment

Brian Wallach\textsuperscript{1}, Julia Clark\textsuperscript{2}
\textsuperscript{1}IAMALS
\textsuperscript{2}Ipsos

PLS


Christina Fournier\textsuperscript{1,2}, Colin Quinn\textsuperscript{3}, Lauren Elman\textsuperscript{3}, Meraida Polak\textsuperscript{2}, Michael Goulbourne\textsuperscript{2}, Jonathan Glass\textsuperscript{2}
\textsuperscript{1}Atlanta VA Medical Center, Atlanta, USA. \textsuperscript{2}Emory University, Atlanta, USA. \textsuperscript{3}University of Pennsylvania, Philadelphia, USA

110. Natural History of PLS and UMN-Predominant ALS in a Large Cohort

Colin Quinn, MD\textsuperscript{1}, Corey T. McMillan, PhD\textsuperscript{1}, Christina Fournier, MD, MSc\textsuperscript{2}, Lauren Elman, MD\textsuperscript{1}
\textsuperscript{1}University of Pennsylvania, Philadelphia, USA. \textsuperscript{2}Emory University, Atlanta, USA

Platform Presentations

The Influence of Clinical Study Inclusion Criteria on Baseline Characteristics and Disease Progression in Amyotrophic Lateral Sclerosis

Jonathan Katz\textsuperscript{1}, Johnna Perdrizet\textsuperscript{2}, Stephen Apple\textsuperscript{2}, Jeffrey Zhang\textsuperscript{3}, Peter Lu\textsuperscript{3}, Wendy Agnese\textsuperscript{2}
\textsuperscript{1}Department of Neurology, Forbes Norris MDA/ALS Center, California Pacific Medical Center, San Francisco, USA. \textsuperscript{2}Mitsubishi Tanabe Pharma America, Inc. (MTPA), Jersey City, USA. \textsuperscript{3}Princeton Pharmatech, Princeton, USA
Motor Cortex Blood-Brain Barrier Opening in Amyotrophic Lateral Sclerosis using MR-Guided Focused Ultrasound: A First-in-Human Trial

Agessandro Abrahao¹, Ying Meng¹,²,³, Maheleth Llinas³, Yuexi Huang⁵, Clement Hamani⁴,²,³, Todd Mainprize⁴, Isabelle Aubert²,⁶, Chinthaka Heyn⁷,⁸, Sandra E. Black¹,²,⁹, Kullervo Hynynen⁸,¹⁰,⁹, Nir Lipsman⁴,²,³, Lorne Zinman¹,²
¹Division of Neurology, Department of Medicine, Sunnybrook Health Sciences Centre, University of Toronto, Toronto, Canada. ²Hurvitz Brain Sciences Research Program, Sunnybrook Research Institute, Toronto, Canada. ³Harquail Centre for Neuromodulation, Sunnybrook Research Institute, Toronto, Canada. ⁴Division of Neurosurgery, Sunnybrook Health Sciences Centre, University of Toronto, Toronto, Canada. ⁵Sunnybrook Research Institute, Toronto, Canada. ⁶Department of Laboratory Medicine and Pathobiology, University of Toronto, Toronto, Canada. ⁷Department of Medical Imaging, Sunnybrook Health Sciences Centre, University of Toronto, Toronto, Canada. ⁸Odette Cancer Research, Sunnybrook Research Institute, Toronto, Canada. ⁹Institute of Biomaterials and Biomedical Engineering, University of Toronto, Toronto, Canada. ¹⁰Department of Medical Biophysics, University of Toronto, Toronto, Canada

Tocilizumab is safe, well-tolerated and reduces C-reactive protein in the plasma and CSF of ALS subjects.

Shafeeq Ladha¹, Phonepasong Arounleut², Nazem Atassi³,⁴, Suma Babu³, Robert Bowser¹, James Caress², Merit Cudkowicz³, Armineuz Evora³, Gregory Hawkins², Tina Kovalik¹, Eric Macklin³, Carol Milligan², Jeremy Shefner¹, Zachary Simmons⁵, Alex Starr¹, Richard Barohn⁶
¹Barrow Neurological Institute, Phoenix, USA. ²Wake Forest University, Winston-Salem, USA. ³Massachusetts General Hospital, Boston, USA. ⁴Sanofi, Boston, USA. ⁵Penn State Hershey Medical Center, Hershey, USA. ⁶Kansas University, Kansas City, USA

Neurofilament Levels in a Multiple Dose Study of a SOD1 Antisense Oligonucleotide (Tofersen) in Participants with ALS

Timothy Miller¹, Merit Cudkowicz², Pamela Shaw³, C. Frank Bennett⁴, Ivan Nestorov⁵, Laura Fanning⁵, Ih Chang⁵, Manjit McNeill⁶, Stephanie Fradette⁵, Toby Ferguson⁵, Yingying Liu⁵, Weiping Chen⁵, Danielle Graham⁵
¹Department of Neurology, Washington University School of Medicine, St. Louis, MO, USA. ²Healey Center, Massachusetts General Hospital, Harvard Medical School, Boston, MA, USA. ³Sheffield Institute for Translational Neuroscience and NIHR Sheffield Biomedical Research Centre, University of Sheffield, Sheffield, United Kingdom. ⁴Ionis Pharmaceuticals, Carlsbad, CA, USA. ⁵Biogen, Cambridge, MA, USA. ⁶Biogen, Maidenhead, United Kingdom

Longitudinal measures of chitinase proteins in ALS and expression of CHI3L1 in activated astrocytes

Lucas Vu¹, Jiyan An¹, Tina Kovalik¹, Tania Gendron², Leonard Petrucelli², Robert Bowser¹
¹Barrow Neurological Institute, Phoenix, USA. ²Mayo Clinic, Jacksonville, USA
Partitioning the genetic architecture of amyotrophic lateral sclerosis

Iris Broce1, Chun Chieh Fan2, Merit Cudkowicz3, Sabrina Paganoni3, Ole Andreassen4, Anders Dale2, Leo Sugrue1, Celeste Karch5, Bruce Miller1, Rahul Desikan1
1University of California, San Francisco, San Francisco, USA. 2University of California, San Diego, San Diego, USA. 3Massachusetts General Hospital, Boston, USA. 4University of Oslo, Oslo, Norway. 5Washington University in St Louis, St Louis, USA

Pathogenic ATXN2 repeat expansions are as common as TARDBP mutations in large ALS cohorts

Cristiane Moreno1,2, Benjamin Hoover1,2, Marie Likanje1,2, Helen Mejia-Santana1,2, Hemali Phatnani3, Rosa Rademakers4,5, Summer Gibson6,2, Daragh Heitzman7,8, Rick Bedlack9,8, Joanne Wuu10,5, Volkan Granit10,5, Jeffrey Statland11,5, Jeff Rothstein12,13, Michael Benatar10,5, Hiroshi Mitsumoto1,8, Matthew Harms1,2
1Columbia University, New York, USA. 2GTAC Study, New York, USA. 3New York Genome Center, New York, USA. 4Mayo Clinic, Jacksonville, USA. 5Project Create, Miami, USA. 6University of Utah, Salt Lake City, USA. 7Texas Neurology, Dallas, USA. 8COSMOS Study, New York, USA. 9Duke University, Durham, USA. 10University of Miami, Miami, USA. 11Kansas University Medical Center, Kansas City, USA. 12Johns Hopkins, Baltimore, USA. 13AnswerALS, Baltimore, USA

Comprehensive analysis of ALS genes in a 5000 patient cohort: gene-specific mutation burdens and challenges of variant classification

Cristiane Moreno1, Danielle Leighton2, Sahar Gelfman1, David Goldstein1, Hemali Phatnani3,4, Matthew Harms1
1Columbia University, New York, USA. 2University of Edinburgh, Edinburgh, United Kingdom. 3New York Genome Center, New York, USA. 4NYGC ALS Consortium, New York, USA

Clinical and genetic characterization of primary lateral sclerosis (PLS): patient registry and whole exome sequencing (WES) *2nd Annual Upper Motor Neuron (UMN) Achievement Award

Nailah Siddique1, Anthony Griswold2, Grace Carlson-Lund1, Kushtrim Ahmeti1, Teepu Siddique1
1Northwestern University Feinberg School of Medicine, Chicago, USA. 2University of Miami Miller School of Medicine, Miami, USA