

Abstracts from the 18th Annual NEALS Meeting

Poster Presentations

4:00pm – 5:00pm: Group A (odd numbers)

5:00 pm – 6:00pm: Group B (even numbers)

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 Peric^{1,2}, Edward Xie¹, Nuran Kocak¹, Mukesh Gautam¹, Johnatan Brent¹, Nicholas Angeloni³, Shad Thaxton^{3,4}, Hande Ozdinler^{1,5,6,7}

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 mitautophagy, a unique self-destructive path, very early in ALS

Mukesh Gautam¹, Edward Xie¹, Nuran Kocak¹, Hande Ozdinler^{1,2,3,4}

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 Ozdinler^{1,3}

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, Hande Ozdinler¹

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

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. ²*Co-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, Robin Redwood, 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-Atienzar¹, 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 Li^{1,2}, Fernande Freyermuth^{1,2}, Nibha Mishra^{1,2}, Yi Han¹, Corey Aguilar¹, Samia Pratt¹, Scott Mordecai³, Jin Kim¹, Patricia Rogers¹, Michael Workman^{1,2}, Ricardos Tabet^{1,2}, Chao Lee^{1,2}, Kitty Savage^{1,2}, Melanie Jambeau^{1,2}, Philip Damme⁴, Kathryn Swoboda¹, Roy Soberman⁵, James Berry¹, Doo Kim¹, Anne Bang⁶, Clotilde Lagier-Tourenne^{1,2}
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,

USA. ³Department of Pathology, Massachusetts General Hospital and Harvard Medical School, Boston, USA.

⁴University of Leuven, Leuven, Belgium. ⁵Department of Medicine, Massachusetts General Hospital and Harvard Medical School, Boston, USA. ⁶Sanford Burnham Prebys Medical Discovery Institute, La Jolla, USA

Biomarker

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 Nicholson¹, Isabel Anez-Bruzual¹, Katherine Burke¹, Diane Lucente¹, Maggie Clapp², Jennifer Jockel-Balsarotti², Amber Malcolm², Taylor Stirrat¹, Lindsay Pothier¹, Tania Gendron³, Mercedes Prudencio³, James Chan⁴, Leonard Petrucelli³, James Berry¹, Timothy Miller¹

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

19. Biomarker analysis in oral levosimendan phase 2 clinical trial LEVALS

Alex M Dickens¹, Ammar Al-Chalabi^{2,3}, Pamela Shaw⁴, P Nigel Leigh⁵, Leonard van den Berg⁶, Orla Hardiman⁷, Albert Ludolph⁸, Valtteri Aho¹, Toni Sarapohja¹, Elina Serkkola¹, Chris Garratt⁹, Kira M Holmström¹

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. ⁷Academic Unit of Neurology, Trinity Biomedical Sciences Institute, Trinity College Dublin, Dublin, Ireland. ⁸Department of Neurology, University of Ulm, Ulm, Germany. ⁹Orion Pharma, Orion Corporation, Nottingham, United Kingdom

20. NEALS Biorepository: A "Living Library"

Dario Gelevski¹, Miriam Moscovitch-Lopatin¹, Alanna Farrar¹, Isabel Anez¹, Cassandra Lieberman¹, Lizzi Neylon², Tina Kovalik², Tara Lincoln³, Terry Heiman-Patterson⁴, Robert Bowser², James Berry¹

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

21. Cognitive Navigation: An Eye-Tracking and Facial Expression Analysis Instrument for the Measurement of Cognitive and Behavioral Abilities in Amyotrophic Lateral Sclerosis

Eufrosina Young^{1,2}, David Mclain³, Lauren Warren-Faricy¹, Claudine Ward¹, Susama Verma², Steven Brose², Servatius Richard²

¹SUNY Upstate, Syracuse, NY, USA. ²Veterans Administration, Syracuse, NY, USA. ³SUNY Oswego, Syracuse, NY, USA

Bulbar

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

Kendrea Garand¹, Debra Suiter², Stephanie Reyes³, Justine Allen⁴, Michelle Moore⁵, Amy Chen⁵

¹University of South Alabama, Mobile, USA. ²University of Kentucky, Lexington, USA. ³Augusta University, Augusta, USA. ⁴University of Florida, Gainesville, USA. ⁵Medical 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 McElheny¹, Jennifer Chapin¹, Lauren DiBiase¹, Amber Anderson¹, Lauren Tabor², James Wymer¹, Emily Plowman¹

¹University of Florida, Gainesville, USA. ²Phil 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 Connaghan¹, Harli Weber², Jordan Green¹, Sabrina Paganoni^{3,2}, James Chan⁴, Ella Collins², Brian Richburg¹, Marziye Eshghi¹, JP Onnela⁵, James Berry^{3,2}

¹MGH Institute of Health Professions, Boston, USA. ²Neurological Clinical Research Institute, Department of Neurology, Massachusetts General Hospital, Boston, USA. ³Harvard Medical School, School of Medicine, Boston, USA. ⁴Massachusetts General Hospital Department of Biostatistics, Boston, USA. ⁵T.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, Raele 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 Hahn¹, Gabriela Stegmann¹, Visar Berisha^{1,2}, Julie Liss^{1,2}, Bettina Cockroft³, Fady Malik³, Lisa Meng³, Stacy Rudnicki³, Andrew Wolff³, Jeremy Shefner⁴

¹Aural Analytics, Scottsdale, USA. ²Arizona State University, Tempe, USA. ³Cytokinetics, South San Francisco, USA. ⁴Barrow 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 Waito^{1,2}, Jordan R Green³, Carolina Barnett Tapia^{2,4}, Rosemary Martino^{2,4}, Agessandro Abrahao¹, Lorne Zinman^{1,2}, Yana Yunusova^{2,1,4}

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 Waito^{1,2}, Chelsea Tanchip², Cindy Cui¹, Reeman Marzouqah^{1,2}, Carolina Barnett Tapia^{2,3}, Agessandro Abrahao¹, Lorne Zinman^{1,2}, Jordan R Green⁴, Yana Yunusova^{2,1,3}

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, MD^{1,2,3}, 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^{1,2}, Anne Haulman², Susan Walsh^{1,2}, Zachary Simmons²

¹ALS Association Greater Philadelphia Chapter, Harrisburg, USA. ²Penn 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^{1,2}, Fabiola De Marchi¹, Amy Ellrodt¹, Michael Doyle¹, Megha Koul¹, Olivia Comeau¹, Elizabeth Adelson¹, Rebecca Walter², Melissa Kusy², Flor Amaya², Carissa Anderson², Jennifer Honda², James Chan³, James Berry¹, Sabrina Paganoni^{1,4,5}

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⁴Department of Physical Medicine and Rehabilitation, Spaulding Rehabilitation Hospital, Boston, USA. ⁵VA Boston Healthcare System, 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^{1,2}, Elena K Bravver^{1,2}, Urvi G Desai^{1,2}, Navid Jalali^{1,2}, William L Bockenek^{1,2}, Scott S Lindblom^{1,2}

¹Carolinas Neuromuscular / ALS MDA Care Center, Charlotte, USA. ²University 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^{1,2}, Elena K Bravver^{1,2}, Urvi G Desai^{1,2}, Navid Jalali^{1,2}, William L Bockenek^{1,2}, Scott S Lindblom^{1,2}

¹Carolinas Neuromuscular / ALS MDA Care Center, Charlotte, USA. ²University 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 McIllduff², 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 Fournier^{1,2}, Richard Bedlack³, Colin Quinn⁴, James Russell⁵, Diane Beckwith², Kathleen Kaminski¹, William Tyor^{1,2}, Vicki Hertzberg², Virginia James², Meraida Polak², Jonathan Glass²

¹Atlanta VA Medical Center, Atlanta, USA. ²Emory University, Atlanta, USA. ³Duke University, Durham, USA. ⁴University of 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 Revel^{3,4}

¹Department of Neurology, The Agnes Ginges Center for Human Neurogenetics, Hadassah-Hebrew University Medical Center, Jerusalem, Israel. ²Hadassah Clinical Research Center Hadassah-Hebrew University Medical Center, Jerusalem, Israel. ³Neurodegenerative Diseases Department at Kadimastem Ltd, Pinchas Sapir 7, Weizmann Science Park, Nes-Ziona, Israel. ⁴Department of Molecular Genetics, Weizmann Institute of Science, 76100, Rehovot, Israel

69. Design of a Phase 3, Randomised, Placebo-Controlled Trial of oral Arimoclomol in Amyotrophic Lateral Sclerosis (ORARIALS-01)

Claus Sundgreen¹, Thomas Blaettler¹, Richard Bennett¹, Dror Rom², Peter M Andersen³, Joanne Wu⁴, Michael Benatar⁴

¹Orphazyme A/S, Copenhagen, Denmark. ²Prosoft Clinical Inc, Huntingdon Valley, USA. ³Department of Pharmacology and Clinical Neuroscience, Umeå University, Umeå, Sweden. ⁴Department 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 Babu¹, Baileigh Hightower², Nicole Zurcher², Chieh-En Tseng², Catherine Cebulla¹, Danica Sanders¹, Olivia Pijanowski¹, Haruhiko Banno¹, Joanna Dojillo³, James Chan⁴, Kazuko Matsuda³, Mark Gudesblatt⁵, Merit Cudkowicz¹, Jacob Hooker², Nazem Atassi¹

¹Sean M Healey & AMG Center for ALS, Massachusetts General Hospital, Boston, USA. ²A. A. Martinos Center for Biomedical Imaging, Department of Radiology, Massachusetts General Hospital, Boston, USA. ³Medicinova Inc, La Jolla, USA. ⁴Department of Biostatistics, Massachusetts General Hospital, Boston, USA. ⁵South 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 Kern¹, James Berry², Revital Aricha¹, Haggai Kaspi¹, Merit Cudkowicz², Anthony Windebank³, Nathan Staff³, Margaret Ayo Owegi⁴, Yossef S. Levy¹, Chaim Lebovits¹, Robert Brown⁴, Yael Gothelf¹

¹Brainstorm Cell Therapeutics, Petach Tikva, Israel. ²Massachusetts General Hospital, Boston, USA. ³Mayo Clinic, Rochester, USA. ⁴UMass 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

¹Carolinas 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, Carlyne 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, MD⁵, Tina Kovalik⁵, Eric Macklin, PhD³, Carol Milligan, PhD¹, Jeremy Shefner, MD, PhD⁵, 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 Connaghan⁵, Jordan Green⁵, Jukka-Pekka Onnela², Sabrina Paganoni^{6,7}, James Berry¹

¹Neurological Clinical Research Institute, Department of Neurology, Massachusetts General Hospital, Boston, USA. ²T.H. Chan Harvard School of Public Health, Boston, USA. ³Massachusetts General Hospital, Department of Neurology, Boston, USA. ⁴Massachusetts General Hospital Biostatistics Center, Boston, USA. ⁵Speech and Feeding Disorders Lab, MGH Institute of Health Professions, Boston, USA. ⁶Massachusetts General Hospital, Neurological Clinical Research Institute (NCRI), Boston, USA. ⁷Department of Physical Medicine and Rehabilitation, Spaulding Rehabilitation Hospital, Boston, USA

82. NeuroREACH™ Platform for Extended Access Program (EAP) Trials as the Foundation for Clinical Research of the Future

Alexander Sherman^{1,2}, Amanda Podesta¹, Kenneth Faulconer¹, Natalia Tarasenko¹, Jason Walker¹, Hong Yu¹, Derek D'Agostino¹, Merit Cudkowicz^{1,2}

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 Cuna³, 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. Conjugal Amyotrophic lateral sclerosis: coincidence or environmental factors? A report on two couples and review of the literature.

Cuiping Zhao^{1,2}, 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'Reilly^{1,2}, Laurence N. Kolonel³, Loic Le Marchand³, Marjorie L. McCullough⁴, Sabrina Paganoni^{5,6}, Michael A. Schwarzschild^{5,6}, Aladdin H. Shadyab⁷, JoAnn E. Manson^{8,1}, Alberto Ascherio^{1,8}

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 Raymond³, 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 Whitworth¹, Alex Sherman¹, Jason Walker², Ervin Sinani¹, Amanda Nichols¹

¹MGH/NCRI, Boston, USA. ²MGH/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 Cytophraphy based quantification of muscle degeneration in Amyotrophic Lateral Sclerosis

Sudarshan Rangunathan, Laura Bell, Ashley Stokes, Natenael 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 Warner¹, Apostolos Tsouris², Andrew D. Schweitzer², Mona Shahbazi¹, Dale Lange¹

¹Hospital for Special Surgery, New York, USA. ²Weill 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 Shah¹, Kevin Boylan¹, Marka Van Blitterswijk¹, Rosa Rademakers^{1,2}, Otto Pedraza¹, Beth Rush¹, Jany Paulett¹, Janay Caradonna¹, Leonard Petrucelli¹, Bjorn Oskarsson¹

¹Mayo Clinic, Jacksonville, FL, USA. ²University 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 Walsh^{1,2}, 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 Thakore¹, 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 Young^{1,2}, 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

¹Massachusetts General Hospital, Center for Innovations and Bioinformatics of NCRI, Boston, USA.

²Massachusetts General Hospital, Center for Innovations and Bioinformatics of NCRI, Boston, USA

107. Long-term survival in amyotrophic lateral sclerosis within the VA Biorepository Brain Bank

Thor Stein^{1,2}, Keith Spencer¹, Zachariah Foster¹, Nazifa Rauf¹, Derek Collins¹, James Averill³, Sean Walker³, Ian Robey³, Neil Kowall¹, Christopher Brady¹

¹VA Boston Healthcare System, Boston, USA. ²Boston University School of Medicine, Boston, USA. ³Southern Arizona VA Healthcare System, Tucson, USA

108. Understanding & Addressing Barriers to ALS Clinical Trial Enrollment

Brian Wallach¹, Julia Clark²

¹ IAMALS

² Ipsos

PLS

109. An Exploratory Analysis of Baseline Prognostic Factors for Patients Presenting with Pure Upper Motor Neuron (UMN) Disease

Christina Fournier^{1,2}, Colin Quinn³, Lauren Elman³, Meraida Polak², Michael Goulbourne², Jonathan Glass²

¹Atlanta VA Medical Center, Atlanta, USA. ²Emory University, Atlanta, USA. ³University of Pennsylvania, Philadelphia, USA

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

Colin Quinn, MD¹, Corey T. McMillan, PhD¹, Christina Fournier, MD, MSc², Lauren Elman, MD¹

¹University of Pennsylvania, Philadelphia, USA. ²Emory University, Atlanta, USA

Platform Presentations

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

Jonathan Katz¹, Johnna Perdrizet², Stephen Apple², Jeffrey Zhang³, Peter Lu³, Wendy Agnese²

¹Department of Neurology, Forbes Norris MDA/ALS Center, California Pacific Medical Center, San Francisco, USA. ²Mitsubishi Tanabe Pharma America, Inc. (MTPA), Jersey City, USA. ³Princeton Pharmatech, Princeton, USA

Motor Cortex Blood-Brain Barrier Opening in Amyotrophic Lateral Sclerosis using MR-Guided Focused Ultrasound: A First-in-Human Trial

Agessandro Abrahao^{1,2,3}, Ying Meng^{4,2,3}, Maheleth Llinas³, Yuexi Huang⁵, Clement Hamani^{4,2,3}, Todd Mainprize⁴, Isabelle Aubert^{2,6}, Chinthaka Heyn^{7,8}, Sandra E. Black^{1,2,9}, Kullervo Hynynen^{8,10,9}, Nir Lipsman^{4,2,3}, Lorne Zinman^{1,2}

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 Atassi^{3,4}, Suma Babu³, Robert Bowser¹, James Caress², Merit Cudkowicz³, Armineuza 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, Mass 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 Moreno^{1,2}, Benjamin Hoover^{1,2}, Marie Likanje^{1,2}, Helen Mejia-Santana^{1,2}, Hemali Phatnani³, Rosa Rademakers^{4,5}, Summer Gibson^{6,2}, Daragh Heitzman^{7,8}, Rick Bedlack^{9,8}, Joanne Wu^{10,5}, Volkan Granit^{10,5}, Jeffrey Statland^{11,5}, Jeff Rothstein^{12,13}, Michael Benatar^{10,5}, Hiroshi Mitsumoto^{1,8}, Matthew Harms^{1,2}

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.

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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 Phatnani^{3,4}, 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