Member Only Day Agenda

Tuesday October 27, 2026

NEALS Member Only Day

All meetings are held in Opal Sands unless indicated otherwise.

- Open to All Attendees
- Open to NEALS Members *(Training Sessions, Workshops, and Committee Meetings)*
- NEALS Affiliated Trial *Open to NEALS Affiliated Study Teams (Invite Only)*
- Meals, Breaks, and Receptions

8:00 AM - 5:00 PM — Registration — **Ballroom Foyer**

Breakfast on your own

7:30 AM - 12:30 PM — Respiratory Care & Ventilation Committee Workshop — **Opal Sol, Solstice IV**

SPONSORED BY SHIONOGI ZEPHYRx

Leads: *Eufrosina Young, MD (SUNY Upstate Medical University), Benjamin Rix Brooks, MD (Clinical Trials Planning, LLC), John Coleman, MD (Northwestern University), and Lisa Wolfe, MD (Northwestern University)*

Workshop Breakout — **Opal Sol, Solstice III**

10:00 AM - 11:00 AM — Recruitment, Retention, and Experience in ALS Research Committee Meeting — **Beach Breeze**

Committee Chairs: *Lindsay Heyd, BS (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital) and Kelly Gwathmey, MD (Columbia University)*

Upper Motor Neuron Committee Meeting — **Sea Watch**

Committee Chairs: *Hande Özdinler, PhD (Northwestern University) and Ikjae Lee, MD (Columbia University)*

11:00 AM - 12:00 PM — Research Coordinator Committee Meeting — **Sea Side & Sea Shore**

Committee Chairs: *Heidi Runk, CCRC (Penn State Health) and Brandy Quarles, MPH, CCRC (Augusta University)*

11:30 AM - 1:30 PM — Lunch — **Opal Ballroom Foyer**

12:00 PM - 1:00 PM — Palliative Committee Meeting — **Sea Watch**

Committee Chairs: *Kellen Quigg, MD, MSc (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital) and Laura Foster, MD (University of Colorado)*

Imaging Committee Meeting — **Beach View**

Committee Chairs: *Erik Pioro, MD, PhD, FRCPC (University of British Columbia) and Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

12:05 PM - 2:45 PM — Clinical Research Staff Training & Development Workshop — **Opal Sand**

SPONSORED BY NURA BIO

Leads: *Lindsay Heyd, BS (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital), Heidi Runk, CCRC (Penn State Health), and Lia Tamburello, BA (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

1:00 PM - 2:00 PM — Advanced Practice Provider Committee Meeting — **Sea Side**

Committee Chairs: *Jourdan Milliard, MSN, FNP-C (Barrow Neurological Institute), Chelsea Simmons, DNP, FNP-BC (The University of North Carolina at Chapel Hill), and Gabriela Lopes, APRN (Nova Southeastern University)*

Member Only Day Agenda

Tuesday October 27, 2026 CONTINUED

NEALS Member Only Day

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2:00 PM - 3:00 PM

Big Data Committee Meeting
Sand Salons

Committee Chair: *Alex Sherman, MSc (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

Technology in ALS Committee Meeting
Sea Watch

Committee Chairs: *Stephen Johnson, MD (Mayo Clinic Scottsdale) and Katherine Burke, PT, DPT, NCS (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

3:00 PM - 7:30 PM

Outcome Measures Training
Opal Sea

Leads: *Gale Kittle, RN, MPH (Barrow Neurological Institute) and Jourdan Milliard, MSN, FNP-C (Barrow Neurological Institute)*

3:00 PM - 4:00 PM

NEALS Information & Community Education (NICE) Working Group
Opal Sol, Estrella Suite

Committee Chairs: *Christina Fournier, MD, MSc (Emory University) and John Novak, MD (Ohio Health). Closed session, invite only*

Genetic ALS, FTD and Related Phenotypes Committee Meeting
Beach Breeze

Committee Chairs: *Matthew Harms, MD (Columbia University) and Rita Sattler, PhD, MSc (Barrow Neurological Institute)*

3:30 PM - 4:30 PM

Refreshment Break
Ballroom Foyer

4:00 PM - 5:00 PM

Physical Therapists & Occupational Therapists Committee Meeting
Sea Watch

Committee Chairs: *Stephanie Johnson, OTR/L, PhD, MHS (Augusta University) and Michele Weaver, PT, DPT, NCS (Corewell Health)*

Nutrition Committee Meeting
Sand Salon

Committee Chairs: *Andrea Charvet, PhD, RDN, LDN (Nova Southeastern University), Ashley Pagano, MS, RD, CSO, LDN (Thomas Jefferson University), and Polina Vorobeychik, MPH (Washington University ALS Center)*

4:00 PM - 6:00 PM

Site Investigator Training SPONSORED BY AFTD The Association for Frontotemporal Degeneration FIND HELP-SHARE HOPE
Opal Sand

Leads: *Senda Ajroud-Driss, MD (Northwestern University), Christina Fournier, MD, MSc (Emory University), Lindsay Heyd, BS (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital), and Meghan Hall, MS, CCRA (Barrow Neurological Institute)*

5:00 PM - 6:00 PM

Clinical Research Nurse Committee Meeting
Beach Breeze

Committee Chair: *Danica Sanders, RN (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

Respiratory Care & Ventilation Committee Meeting
Sea Shore and Side

Committee Chairs: *John Coleman, MD (Northwestern University), Eufrosina Young, MD (SUNY Upstate Medical University), and Benjamin Rix Brooks, MD (Clinical Trials Planning, LLC)*

Day One Agenda

Wednesday October 28, 2026

General sessions are livestreamed

All meetings are held in Opal Sol unless indicated otherwise.

Livestreamed session

- Open to All Attendees
- Open to NEALS Members *(Training Sessions, Workshops, and Committee Meetings)*
- NEALS Affiliated Trial *Open to NEALS Affiliated Study Teams (Invite Only)*
- Meals, Breaks, and Receptions

7:00 AM - 4:30 PM Registration — **Ballroom Foyer**

7:00 AM - 8:30 AM Breakfast — **Ballroom Foyer**

7:15 AM - 8:15 AM LUMINA Study Meeting — **Equinox I**

VITALS Study Meeting — **Opal Sands, Sand Salon**

7:15 AM - 8:15 AM Global Research & Outreach Working Group Meeting — **Equinox II & III**

8:30 AM - 8:50 AM Opening Session: NEALS Annual Update — **Ballroom**

Jinsy Andrews, MD, MSc (New York University) and Nicholas Maragakis, MD (Johns Hopkins University)

8:50 AM - 10:30 AM The Next Frontier in ALS: Integrating Clinical Care, Research, and Lived Experience for People at Genetic Risk of ALS/FTD — **Ballroom**

Session Lead: *Nicholas Maragakis, MD (Johns Hopkins University)*

8:50 AM Session Introduction
Nicholas Maragakis, MD (Johns Hopkins University)

8:55 AM Presymptomatic ALS/FTD and Disease Prevention: A View from the Vanguard
Michael Benatar, MD, PhD (University of Miami)

9:07 AM Expanding Presymptomatic Studies to a National Scale: the PREVENT ALL ALS Study
Mark Garret, MD (Dartmouth Hitchcock Medical Center)

9:19 AM Supporting the Journey, Empowering Choice: Predictive Genetic Counseling in ALS/FTD
Laynie Dratch, SCM, CGC (Penn Comprehensive ALS Center)

9:31 AM Building the Next Frontier: Connecting Care, Research, and Lived Experience
Cassandra Haddad, MSN (ALS Research Ambassador)

9:43 AM Developing a Care Approach for Individuals at Genetic Risk of ALS/FTD
Terry Heiman-Patterson, MD (Temple University)

9:55 AM Racing Against the Clock: Patient Advocates as Key Architects of the Self Cure
Yentli Soto Albrecht, PhD (University of Pennsylvania)

10:07 AM Moderated Q&A

10:30 AM - 10:50 AM Break SPONSORED BY Acelot — **Ballroom Foyer**

Wednesday October 28, 2026 CONTINUED

General sessions are livestreamed

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Livestreamed session

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- NEALS Affiliated Trial *Open to NEALS Affiliated Study Teams (Invite Only)*
- Meals, Breaks, and Receptions

10:50 AM - 12:25 PM — NEALS Affiliated Trial Session — **Ballroom**

Session Lead: *Jinsy Andrews, MD, MSc (New York University)*

10:50 AM Co-Chair Summary Roundup

11:05 AM Learnings from the Phase 2 Study of AP-101 (AP101-02) and the Role of Weight as a Surrogate Marker of Clinical Response
Angela Genge, MD, FRCPC (AL-S Pharma AG)

11:20 AM Update on ATLAS, a Presymptomatic Interventional Study of Tofersen in SOD1-ALS
Steve Garafalo, PhD (Biogen)

11:35 AM LUMINA: AMX0114 ALS Study Update
Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital) and Lauren Kett, MD, PhD (Amylyx Pharmaceuticals)

11:50 AM Update on ARMOR, a First-in-Human Dose-Escalation Study of INS1202 Gene Therapy in ALS
Mark Stahl, MD, PhD (Insmed)

12:05 PM Seamless Therapy Development in ALS
Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)

12:30 PM - 2:30 PM — Lunch SPONSORED BY ALS INVESTMENT FUND — **Opal Sands, Event Lawn**

For meetings at Opal Sands, lunch will be available on the Mezzanine Level.

12:45 PM - 1:45 PM — PIONEER-ALS Study Meeting — **Equinox I**

HEALEY Platform Trial Study Meeting — **Solstice I and II**
Closed session, invite only

12:45 PM - 3:15 PM — NEALS Bulbar Symposium: Best Practices on Management of Bulbar ALS: From Evidence to Clinical Practice — **Opal Sands, Sand Salons**
Lead: *Gary Pattee, MD (Neurology Associates, P.C.)*

1:00 PM - 3:00 PM — PT/OT Optimizing Function Across the ALS Continuum: PT & OT Considerations for Clinical Practice — **Opal Sands, Beach Salons**
Leads: *Stephanie Johnson, OTR/L, PhD, MHS (Augusta University) and Michele Weaver, PT, DPT, NCS (Corewell Health)*

Wednesday October 28, 2026 CONTINUED

General sessions are livestreamed

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Livestreamed session

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2:00 PM - 3:00 PM

ALL ALS Study Meeting — **Equinox II & III**

Healey MyMatch Study Meeting — **Opal Sands, Sea Salons**
Closed session, invite only

2:00 PM - 3:15 PM

Lightning Science Talks — **Ballroom**

Session Leads: *Sami Barmada, MD, PhD (University of Michigan) and Jeffrey Rothstein, MD, PhD (Johns Hopkins University)*

2:00 PM Welcome and Introductions

2:05 PM Science Presentations, Three Minutes Each

DPTX3230 Restores TDP-43 Nuclear Function Across Translational Models of ALS
Isaac Klein, MD, PhD (Dewpoint Therapeutics)

RNA Processing Dysregulation and Fusion Transcript Formation in ALS
Ghazaleh Sadri-Vakili, PhD, MS (Massachusetts General Hospital)

A Harmonized Multisite Digital TDP-43 Pathology Resource Enables Automated Quantitative Analysis Across ALS and FTD
Mayisha Rahman, MS (Georgetown University)

Bypassing the Blood-Brain Barrier: Systemic Delivery of SYT2-Targeted siSOD1 to Lower Motor Neurons Attenuates Motor Decline in SOD1-G93A Mice
Norihiro Yumoto, PhD (Jiksak Bioengineering)

2:17 PM Group Q&A

2:25 PM Transition

2:28 PM Clinical Presentations, Group One, Three Minutes Each

ERACE (Emory Rapid Access Clinical Evaluation) 10-Protein Model Predicts ALS from Plasma with High Accuracy
Eleanor Thomas, MD, PhD (Emory University)

Advancement of BIIB147, an Antisense Oligonucleotide Designed to Correct STMN2 Splicing, to a First-in-Human Phase 1/2 Study in ALS
Steve Garafalo, PhD (Biogen)

Long-term (5 years) Evaluation of Tofersen in SOD1-ALS: Final Results from the Integrated Analysis of Study 101 Parts A/B, a Phase 1/2 Ascending Dose Trial, and the Open-Label Extension
Timothy Miller, MD, PhD (Washington University ALS Center)

Propensity-Matching Analysis Indicates that RAPA-501 Autologous T Stem Cell Therapy Mediates Promising Clinical Benefits in Advanced-Stage ALS Patients
James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)

2:40 PM Group Q&A

2:48 PM Transition

Wednesday October 28, 2026 CONTINUED

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2:00 PM - 3:15 PM — Lightning Science Talks, continued — **Ballroom**

Session Leads: *Sami Barmada, MD, PhD (University of Michigan) and Jeffrey Rothstein, MD, PhD (Johns Hopkins University)*

2:51 PM Clinical Presentations, Group Two, Three Minutes Each

GLP-1 Receptor Agonist Treatment is Associated with Shorter Survival in People with Amyotrophic Lateral Sclerosis and Comorbid Diabetes Mellitus
Ikjae Lee, MD (Columbia University)

Healey ALS MyMatch: Program Overview and Latest Trial Updates
Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)

Tim Lowrey ALS Panels: Lived Experience ALS Stories for Health-Care Students and Professionals
Timothy Abeska, JD (I AM ALS Volunteer Advocate)

Real-Time Communication Restoration in ALS with a Fully Implanted Speech Brain-Computer Interface
Nader Pouratian, MD, PhD (UT Southwestern Medical Center)

3:03 PM Group Q&A

3:11 PM Closing Remarks

3:15 PM - 3:30 PM — Break — **Ballroom Foyer**

3:30 PM - 4:30 PM — Industry Roundtable: Advancing TDP-43-Relevant Therapeutic Targets — **Ballroom**

Session Lead: *Nicholas Maragakis, MD (Johns Hopkins University)*

Irina Antonijevic, MD, PhD (Trace Neuroscience), Martin Engel, PhD (Neurizon), Isaac Klein, MD, PhD (Dewpoint Therapeutics), Katie Planey, PhD, MBA (Acelot), and Olga Uspenskaya, MD, PhD (VectorY Therapeutics)

4:30 PM - 6:30 PM — Poster Session 1 SPONSORED BY SHIONOGI — **Opal Sands, Ballroom**

5:30 PM - 7:00 PM — The Palliative Story Exchange — **Opal Sands, Beach Salon**

External event for NEALS members.

Session Leads: *Richard Leiter, MD, MA (The Palliative Story Exchange) and Lisa Keefauver, MSW (The Palliative Story Exchange)*

Day Two Agenda

Thursday October 29, 2026

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- NEALS Affiliated Trial *Open to NEALS Affiliated Study Teams (Invite Only)*
- Meals, Breaks, and Receptions

7:00 AM - 4:15 PM Registration — **Ballroom Foyer**

7:00 AM - 8:30 AM Breakfast — **Ballroom Foyer**

7:15 AM - 8:15 AM ALSTARS Study Meeting — **Equinox I**

PREVAiLS Study Meeting — **Opal Sands, Sand Salons**

7:15 AM - 8:15 AM CDC Informational Session: How the National ALS Registry Can Support Your Research — **Equinox II and III**

Session Lead: *Paul Mehta, MD (Centers for Disease Control and Prevention)*

8:30 AM - 9:30 AM Embedding the ALS Patient Journey into Clinical Development, Care Delivery, and Advocacy — **Ballroom**

Session Lead: *Michael Robinson, MD (ALS Research Ambassador)*

Mandi Bailey (ALS Research Ambassador), William Benjamin Nowell, PhD, MSW (Regeneron), John Driskell Hopkins (Hop On A Cure), and Christina Fournier, MD, MSc (Emory University)

9:30 AM - 9:50 AM Break — **Ballroom Foyer**

9:50 AM - 10:50 AM A Collaborative Ecosystem for Clinical Trial Innovation — **Ballroom**

Session Lead: *Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

9:55 AM Early Incorporation of Lived Experience in the Prosetin Development Program
Erin Fleming, BA (ProJenX)

10:05 AM Rapid Bench-to-Bedside Translation of Digoxin in ALS: the Healey ALS MyMatch ACACIA trial
Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital) and Brian Wainger, MD, PhD (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)

10:15 AM *Jinsy Andrews, MD, MSc (New York University)*

10:25 AM Natural History Studies to Drive Research Innovation & Clinical Trial Interpretation
Robert Bowser, PhD (Barrow Neurological Institute)

10:35 AM Implantable Brain-Computer Interfaces (iBCIs)
Leigh Hochberg, MD, PhD (Massachusetts General Hospital)

10:55 AM - 11:55 AM Stats Corner: NfL — The Biomarker That Rocks Every Role — **Ballroom**

Session Lead: *Lori Chibnik, PhD, MPH (Massachusetts General Hospital Biostatistics)*

11:00 AM *Eric Macklin, PhD (Massachusetts General Hospital Biostatistics)*

11:05 AM *Jianing Wang, PhD (Massachusetts General Hospital Biostatistics)*

11:10 AM *Giorgio Paulon, PhD (Berry Consultants)*

11:15 AM *Peng Sun, PhD (Biogen)*

11:20 AM Moderated Panel Discussion and Q&A

Thursday October 29, 2026 CONTINUED

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- NEALS Affiliated Trial *Open to NEALS Affiliated Study Teams (Invite Only)*
- Meals, Breaks, and Receptions

11:55 AM - 1:00 PM Lunch SPONSORED BY PACKARD CENTER — **Opal Sands, Event Lawn**

For meetings at Opal Sands, lunch will be available on the Mezzanine Level.

12:00 PM - 1:00 PM ARMOR Study Meeting — **Equinox I**

Clene NIH – Expanded Access Program Meeting — **Opal Sands, Beach Salons**

12:00 PM - 1:00 PM NEALS Biorepository Informational Session — **Equinox II and III**

Session Leads: *James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital) and Ikjae Lee, MD (Columbia University)*

12:00 PM - 1:00 PM Networking Lunch: Sustaining Multidisciplinary Clinics: Funding Challenges and Strategic Solutions — **Opal Sands, Sand Salons**

NEALS members. Lunch for networking sessions on the mezzanine.

Networking Lunch: Mentorship and Career Growth with Senior Researchers — **Opal Sands, Sea Salon**

NEALS members. Lunch for networking sessions on the mezzanine.

1:10 PM - 1:30 PM Co-Chair Announcement — **Ballroom**

Jinsy Andrews, MD, MSc (New York University) and Nicholas Maragakis, MD (Johns Hopkins University)

1:30 PM - 3:30 PM Science Symposium: AI in ALS: From Bench Insights to Bedside Impact — SPONSORED BY Biogen — **Ballroom**

Session Leads: *Sami Barmada, MD, PhD (University of Michigan), Timothy Miller, MD, PhD (Washington University ALS Center), and Jeffrey Rothstein, MD, PhD (Johns Hopkins University)*

1:55 PM Decoding ALS with AI, From Neuron to Brain Bank: Phenotypic Signatures, Mechanism, and TDP-43 Pathology at Scale
Steve Finkbeiner, MD, PhD (Gladstone Institutes)

2:20 PM Connecting the Clues in ALS: An Investigation Across Datasets
Juro Gottweis (Independent Researcher) and Luka Rimanic, PhD (Google)

2:45 PM Early Identification of ALS Using Advanced Machine Learning and Real-World Data
Vahid Esmaeili, PhD (Volv Global) and Christophe Lo Bianco, PhD (Novartis)

3:10 PM SAVA AI: A Collaborative Platform Connecting Clinicians, Sites, Sponsors, and Patients to Enable Real-Time Clinical Trial Matching
Natalia Luchkina, PhD (EverythingALS) and Anusha Rao (EverythingALS)

3:20 PM A Variant-Anchored, Multi-Agent AI Method for Designing RNA-Targeting Strategies in TDP-43 Proteinopathy: A Worked Example in TARDBP F231V
Richard Broadbent (InnerVara Nexus AI Solutions)

Day Two Agenda

Thursday October 29, 2026 CONTINUED

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Livestreamed session

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- Meals, Breaks, and Receptions

3:30 PM - 3:50 PM	Break SPONSORED BY Acelot	Ballroom Foyer
3:50 PM - 4:50 PM	Hot Topic Session: Causal Inference in ALS: What are the Data for Environmental Exposures **Session Lead:** *Jonathan Glass, MD (Emory University)* *Stephen Goutman, MD, MS, FAAN (University of Michigan), Björn Oskarsson, MD (Mayo Clinic), and Marc Weisskopf, PhD, ScD (Harvard T.H. Chan School of Public Health)*	Ballroom
4:50 PM - 6:30 PM	Poster Session 2	Opal Sands, Ballroom
6:30 PM - 6:45 PM	Group Photo	Opal Sands, Lobby
6:45 PM - 9:00 PM	Annual Beachside Dinner SPONSORED BY ALS ASSOCIATION	Opal Sands, Event Lawn

Day Three Agenda

Friday October 30, 2026

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Livestreamed session

- Open to All Attendees
- Open to NEALS Members *(Training Sessions, Workshops, and Committee Meetings)*
- NEALS Affiliated Trial *Open to NEALS Affiliated Study Teams (Invite Only)*
- Meals, Breaks, and Receptions

7:00 AM - 12:30 PM Registration — **Ballroom Foyer**

7:00 AM - 8:30 AM Breakfast — **Ballroom Foyer**

7:15 AM - 8:15 AM FUNCtion ALS Study Meeting — **Equinox II**

LINK Study Meeting — **Equinox III**

8:30 AM - 9:35 AM Lessons from Novel Therapies: Beyond ALS — **Ballroom**

Session Lead: *Nicholas Maragakis, MD (Johns Hopkins University)*

8:35 AM New Approaches to Treating Duchenne Muscular Dystrophy
Kevin Flanigan, MD (Ohio State University)

9:05 AM Evolving Treatments for SMA: Insights for ALS
Charlotte Sumner, MD (Johns Hopkins University)

9:40 AM - 10:15 AM The Power of Partnership: Updates from the NEALS ALS Organizations Forum — **Ballroom**

Session Lead: *Jinsy Andrews, MD, MSc (New York University)*
Kuldip Dave, PhD (The ALS Association), Kristen Cocoman (ALS United), and Terry Heiman-Patterson, MD (ALS Hope Foundation)

10:15 AM - 10:30 AM Break — **Ballroom Foyer**

10:30 AM - 10:50 AM Awards — **Ballroom**

Session Lead: *Jinsy Andrews, MD, MSc (New York University)*

The Gupta Family Endowed Prize for Innovation in ALS Care
Presented by: *Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

Drs. Ayeez and Shelena Lalji & Family Student Scholar Award for Repair and Regenerative Mechanisms in ALS
Presented by: *Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)*

10:50 AM - 11:05 AM Abstract Awards — **Ballroom**

Session Lead: *Terry Heiman-Patterson, MD (Temple University)*

Basic Science Award
Presented by: *Terry Heiman-Patterson, MD (Temple University)*

Clinical Investigation Award
Presented by: *Terry Heiman-Patterson, MD (Temple University)*

Upper Motor Neuron Achievement Awards
Presented by: *Christine Hendrickson, ARNP (Spastic Paraplegia Foundation Ambassador)*

Day Three Agenda

Friday October 30, 2026 CONTINUED

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- Meals, Breaks, and Receptions

11:05 AM - 12:50 PM Platform Presentations — **Ballroom**

Session Lead: *Terry Heiman-Patterson, MD (Temple University)*

11:10 AM Novel Small Molecule is First-in-Class to Bind to FUS and Reverse Loss and Gain of Function
Baerbel Rohrer, PhD (Medical University of South Carolina)

11:25 AM From PARADIGM to PARAGON: Converging Clinical and Biomarker Findings Informing Phase 3 Development of PrimeC
Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)

11:40 AM ACE-2223, a First-in-class Oral Small Molecule for Sporadic ALS, Treats Both Gain- and Loss-of-function TDP-43 Pathology, Extends Survival and Decreases NfL in ALS Mouse Models and has Begun Human Safety Studies
Mark Forman, MD, PhD (Acelot)

11:55 AM Genome-wide Screening of Nuclear Import Modulation in Human Neurons Identifies Genetic Pathways that Sustain Nuclear TDP-43: ALS and Beyond
Jeffrey Rothstein, MD, PhD (Johns Hopkins University)

12:10 PM Metformin Shows Favorable Safety and Reduces RAN Proteins, Markers of Neurodegeneration (NfL) and Neuroinflammation in C9orf72 ALS Patients in a Phase II Open-label Clinical Trial
Laura Ranum, PhD (University of Florida)

12:25 PM EPISOD1: A Phase 1/2, Multicenter Study to Evaluate the Safety, Tolerability, and Exploratory Efficacy of Intrathecally Administered Gene Therapy AMT-162 in Patients with SOD1 Amyotrophic Lateral Sclerosis (SOD1-ALS)
James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital)

12:40 PM Audience Q&A

12:50 PM - 12:55 PM Closing Remarks — **Ballroom**
Jinsy Andrews, MD, MSc (New York University) and Nicholas Maragakis, MD (Johns Hopkins University)

12:50 PM - 2:00 PM Lunch — **Ballroom Foyer**

1:10 PM - 2:10 PM ALS Organizations Forum — **Equinox II and III**
Closed session