

2026 ANNUAL NEALS MEETING AGENDA*

*Agenda subject to change

MEETING AND BREAKOUT COLOR LEGEND

General Info & General Sessions

Meals & Receptions

(open to all attendees)

NEALS Working Group & Committee Meetings

(open to NEALS members)



NEALS Affiliated Trial: Study

Investigator Meetings

(limited to site investigators & study teams)

NEALS Member Training Sessions & Workshops

(open to NEALS members)

NEALS MEMBER ONLY DAY TUESDAY, OCTOBER 27, 2026

All meetings in Opal Sands unless otherwise noted.

8:00a – 5:00p	Registration (Breakfast on own)	Ballroom Foyer
7:30 – 12:30p	Respiratory Care & Ventilation Committee Workshop Workshop Breakout <i>Moderators: Eufrosina Young, MD (SUNY Upstate Medical University), Benjamin Rix Brooks MD (Clinical Trials Planning, LLC), John Coleman, MD (Northwestern University), Lisa Wolfe, MD (Northwestern University)</i> <i>Presenters: Jason Ackrivo MD, MSCE (University of Pennsylvania), Pamela Cazzolli, RN (ALS Care Project), Philip Choi, MD (NYU Langone Health), Tamara LeBlanc, BSRC-RRT (NSU Health), Won Lee, MD (UT Southwestern), Zachary Simmons, MD (Penn State Health), George Slavinski, RT (SUNY Upstate Medical University), and Kathleen Twomey, MD (Penn State Health)</i>	(Opal Sol) Solstice IV (Opal Sol) Solstice III
10:00 – 11:00a	Recruitment, Retention, and Experience in ALS Research Committee Meeting <i>Committee Chairs: Lindsay Heyd, BS (Sean M. Healey & AMG Center for ALS at MGH) and Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH)</i>	Beach Breeze
10:00 – 11:00a	Upper Motor Neuron Committee Meeting <i>Committee Chairs: Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH), Jinsy Andrews, MD, MSc (NYU Langone Medical), and Hande Özdinler, MD (Northwestern University)</i>	Sea Watch
11:00 – 12:00p	Research Coordinator Committee Meeting <i>Committee Chairs: Heidi Runk, CCRC (Penn State) and Brandy Quarles, MPH, CCRC (Augusta University)</i>	Sea Side
11:30 – 1:30p	Lunch	Opal Ballroom Foyer
12:00 – 1:00p	Palliative Committee Meeting <i>Committee Chairs: Ambereen Mehta, MD, MPH, FAAHPM (Johns Hopkins University) and Laura Foster, MD (University of Colorado)</i>	Sea Watch

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NEALS MEMBER ONLY DAY (CONTINUED) TUESDAY, OCTOBER 27, 2026

All meetings in Opal Sands unless otherwise noted.

12:00 – 1:00p	Imaging Committee Meeting <i>Committee Chairs: Erik Pioro, MD, PhD, FRCPC (University of British Columbia) and Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at MGH)</i>	Beach View
12:00 – 2:30p	Clinical Research Staff Training & Development Workshop <i>Leads: Lindsay Heyd (Sean M. Healey & AMG Center for ALS at Mass General), Heidi Runk, CCRC (Penn State), Lia Tamburello (Sean M. Healey & AMG Center for ALS at Mass General)</i> <i>David Buseck (PALS Advisor to the NEALS Executive Committee), Jayna Duell, BSN, RN (University of Michigan), Kelly Gwathmey, MD (Columbia University), Nicholas Maragakis, MD (Johns Hopkins)</i>	Opal Sand
1:00 – 2:00p	Advance Practice Provider Committee Meeting <i>Committee Chairs: Jourdan Milliard, MSN, FNP-C (Barrow Neurological Institute), JinAe Arneklev, MSN, CRNP (Johns Hopkins University), and Gabriela Lopes, APRN (Nova Southeastern University)</i>	Sea Side
2:00 – 3:00p	Big Data Committee Meeting <i>Committee Chair: Alexander Sherman, MS (Sean M. Healey & AMG Center for ALS at MGH)</i>	Sand Salons
2:00 – 3:00p	Technology in ALS Committee Meeting <i>Committee Chairs: Andrew Geronimo, PhD (Penn State), Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH) and Stephen Johnson, MD (Mayo Clinic Scottsdale)</i>	Sea Watch
3:00 – 7:30p	Outcome Measures Training <i>Lead: Gale Kittle, RN, MPH (Barrow Neurological Institute) and Jourdan Milliard, FNP-C (Barrow Neurological Institute)</i>	Opal Sea
3:00 – 4:00p	NEALS Information & Community Education (NICE) Working Group (Closed Session; Invite Only)	(Opal Sol) Estrella Hospitality Suite
3:00 – 4:00p	Genetic ALS, FTD and Related Phenotypes Committee Meeting <i>Committee Chairs: Claire Flaherty-Craig, PhD, MS (Penn State), Matthew Harms, MD (Columbia University), and Rita Sattler, PhD (Barrow Neurological Institute)</i>	Beach Breeze
3:30 – 4:30p	Refreshment Break	Ballroom Foyer
4:00 – 5:00p	Physical Therapists & Occupational Therapists Committee Meeting <i>Committee Chairs: Stephanie Johnson OTR/L PhD MHS (Augusta University), and Michele Weaver, PT, DPT, NCS (Corewell Health)</i>	Sea Watch

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NEALS MEMBER ONLY DAY (CONTINUED) TUESDAY, OCTOBER 27, 2026

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4:00 – 5:00p	Nutrition Committee Meeting <i>Committee Chairs: Andrea Charvet, PhD, RDN, LDN (Nova Southeastern University), Stephanie Dobak, MS, RD, LDN (Jefferson Weinberg ALS Center), and Polina Vorobeychik, MPH (Washington University ALS Center)</i>	Sand Dune
4:00 – 6:00p	Site Investigator Training <i>Leads: Senda Ajroud-Driss, MD (Northwestern University), Christina Fournier, MD (Emory School of Medicine), Lindsay Heyd (Sean M. Healey & AMG Center for ALS at MGH), and Meghan Hall, MS, CCRA (Barrow Neurological Institute)</i>	Opal Sand
5:00 – 6:00p	Clinical Research Nurse Committee Meeting <i>Committee Chairs: Danica Sanders, RN (Sean M. Healey & AMG Center for ALS at MGH)</i>	Beach Breeze
5:00 – 6:00p	Respiratory Care & Ventilation Committee Meeting <i>Committee Chairs: Christopher D. Lee, MD, MPH (Vanderbilt University), Eufrosina Young, MD (SUNY Upstate) and Benjamin Rix Brooks, MD (Clinical Trials Planning LLC)</i>	Sea Shore & Side

(End of NEALS Member Only day)

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Investigator Meetings

(limited to site investigators & study teams)

NEALS Member Training Sessions & Workshops

(open to NEALS members)

DAY ONE WEDNESDAY, OCTOBER 28, 2026

All meetings in Opal Sol unless otherwise noted.

7:00a – 4:30p	Registration	Ballroom Foyer
7:00 – 8:30a	Breakfast	Ballroom Foyer
7:15 – 8:15a	LUMINA Study Meeting	Equinox I
7:15 – 8:15a	Global Research & Outreach Working Group Meeting	TBD
8:30 – 8:50a	Opening Session <i>Jinsy Andrews, MD, MSc (NYU Langone Medical)</i> <i>Nicholas Maragakis, MD (Jons Hopkins)</i>	Ballroom
8:50 – 10:30a	The Next Frontier in Amyotrophic Lateral Sclerosis (ALS): Integrating Clinical Care, Research, and Lived Experience for People at Genetic Risk of ALS/FTD <i>Moderator: Nicholas Maragakis, MD (Johns Hopkins)</i> Pre-symptomatic ALS/FTD and disease prevention: a view from the vanguard <i>Michael Benatar, MD, PhD (University of Miami)</i> Expanding presymptomatic studies to a national scale: the PREVENT ALL ALS study <i>Mark Garret, MD (Dartmouth Hitchcock Medical Center)</i> Supporting the Journey, Empowering Choice: Predictive Genetic Counseling in ALS/FTD <i>Laynie Dratch, SCM, CGC (Penn Comprehensive ALS Center)</i> Building the Next Frontier: Connecting Care, Research, and Lived Experience <i>Cassandra Haddad, MSN (ALS Research Ambassador)</i> Developing a care approach for individuals at genetic risk of ALS/FTD <i>Terry Heiman-Patterson, MD (Temple University)</i>	Ballroom

2026 ANNUAL NEALS MEETING AGENDA*

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DAY ONE (CONTINUED) WEDNESDAY, OCTOBER 28, 2026

All meetings in Opal Sol unless otherwise noted.

8:50 – 10:30a	 The Next Frontier in Amyotrophic Lateral Sclerosis (ALS): Integrating Clinical Care, Research, and Lived Experience for People at Genetic Risk of ALS/FTD (continued) <i>Moderator: Nicholas Maragakis, MD (Johns Hopkins)</i> Racing Against the Clock: Patient Advocates as Key Architects of the Self Cure <i>Yentli Soto Albrecht, PhD (University of Pennsylvania)</i>	Ballroom
10:30 – 10:50a	Break SPONSORED BY 	
10:50 – 12:25p	 NEALS Affiliated Trial Session <i>Session Lead: Jinsy Andrews, MD, MSc (NYU Langone Health)</i>	Ballroom
10:50a	Co-Chair Summary Roundup	
11:05a	Presentations: Study Update 1 <i>Angela Genge, MD, FRCPC (Montreal Neurological Institute-Hospital)</i>	
11:20a	Update on ATLAS, a presymptomatic interventional study of tofersen in SOD1-ALS <i>Steve Garafalo, PhD (Biogen)</i>	
11:35a	LUMINA: AMX0114 ALS Study Update <i>Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH)</i> <i>Lauren Kett, MD, PhD (Amylyx Pharmaceuticals)</i>	
11:50a	Study Update 4	
12:05p	Seamless Therapy Development in ALS <i>Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at MGH)</i>	
12:25 – 2:00p	Lunch	(Opal Sands) Pool Event Lawn
12:45 – 1:45p	VITALS Study Meeting	Solstice IV
12:45 – 1:45p	PIONEER-ALS Study Meeting	Equinox I
12:45 – 1:45p	HEALEY Platform Trial Study Meeting (Closed Session; Invite Only)	Solstice II & III

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DAY ONE (CONTINUED) WEDNESDAY, OCTOBER 28, 2026

All meetings in Opal Sol unless otherwise noted.

12:45 – 3:15p	NEALS Bulbar Symposium: Best Practices on Management of Bulbar ALS: From Evidence to Clinical Practice <i>Lead: Gary Pattee, MD (Neurology Associates, P.C.)</i> <i>John Costello, MA, CCC-SLP (Boston Children's Hospital)</i> <i>Laura Foster, MD (University of Colorado Anschutz)</i> <i>Jackie Klunk, MS, RDN, LDN, (University of Pittsburgh)</i> <i>Kellen Quigg, MD (Massachusetts General Hospital)</i> <i>Benjamin Rix Brooks, MD (Clinical Trials Planning LLC)</i> <i>Kay Stolwyk, MS, RD, CDN (Columbia University)</i> <i>Lauren Tabor Gray, PhD, SLP (NOVA Southeastern University)</i> <i>Yana Yunusova, PhD, CCC-SLP (University of Toronto)</i>	(Opal Sands) Sand Salons
1:00 – 3:00p	PT/OT Optimizing Function Across the ALS Continuum: PT & OT Considerations for Clinical Practice <i>Leads: Stephanie Johnson, OTR/L, PhD, MHS (Augusta University), and Michele Weaver, PT, DPT, NCS (Corewell Health)</i> <i>Matt Abisamra, OTR/L, CDRS (Shepherd Center)</i> <i>Kayla Chomko, PT, DPT, NCS (NOVA Southeastern University)</i> <i>Lauren Nidiffer, PT, DPT (Ohio Health ALS Clinic)</i> <i>Lizbeth Valdez James, MSW (NOVA Southeastern University)</i>	(Opal Sands) Beach Salons
2:00 – 3:00p	ALL ALS Study Meeting	Equinox II & III
2:00 – 3:00p	Healey MyMatch Study Meeting (Closed Session; Invite Only)	Solstice I & II
2:00 – 3:10p	 Lighting Science Talks <i>Session Leads: Science Advisory Board Co-Chairs Sami Barmada, MD, PhD (University of Michigan and Jeffrey Rothstein, MD, PhD (Johns Hopkins University)</i> Science Presentations: 3-Minute Talks DPTX3230 Restores TDP-43 Nuclear Function Across Translational Models of ALS <i>Isaac Klein, MD, PhD (Dewpoint Therapeutics)</i> RNA processing dysregulation and fusion transcript formation in ALS <i>Ghazaleh Sadri-Vakili, PhD, MS (Mass General)</i> Audience Q&A Clinical Presentations: 3-Minute Talks Advancement of BIIB147, an Antisense Oligonucleotide Designed to Correct STMN2 Splicing, to a First-in-Human Phase 1/2 Study in ALS <i>Colin Quinn, MD (University of Pennsylvania)</i>	Ballroom

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DAY ONE (CONTINUED) WEDNESDAY, OCTOBER 28, 2026

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2:00 – 3:10p

 **Lighting Science Talks (continued)**
Session Leads: Science Advisory Board Co-Chairs
Sami Barmada, MD, PhD (University of Michigan and
Jeffrey Rothstein, MD, PhD (Johns Hopkins University)

Clinical Presentations: 3-Minute Talks (continued)

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)

GLP-1-receptor agonist treatment is associated with shorter survival in people with amyotrophic lateral sclerosis and comorbid diabetes mellitus
Ikjae Lee, MD MSc (Columbia University)

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

Audience Q&A

3:15 – 3:30p

Break

3:30 – 4:30p

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

Ballroom

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

Audience Q&A

4:30 – 6:30p

Poster Session 1

SPONSORED BY



(Opal Sands) Ballroom

5:30 – 7:00p

The Palliative Story Exchange (External Event for NEALS Members) (Opal Sands) Beach Salon
Moderators: *Richard Leiter, MD, MA (The Palliative Story Exchange)*
and *Lisa Keefauver, MSW (The Palliative Story Exchange)*

(End of day 1)

2026 ANNUAL NEALS MEETING AGENDA*

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DAY TWO

THURSDAY, OCTOBER 29, 2026

All meetings in Opal Sol
unless otherwise noted.

7:00a – 4:15p	Registration	Ballroom Foyer
7:00 – 8:30a	Breakfast	Ballroom Foyer
7:15 – 8:15a	ALSTARS Study Meeting	Equinox I
7:15 – 8:15a	PREVAiLS Study Meeting	(Opal Sands) Sand Salons
7:15 – 8:15a	CDC Informational Session: How the National ALS Registry can Support your Research <i>Session Lead: Paul Mehta, MD (CDC)</i>	Equinox II & III
8:30 – 9:30a	 Embedding the ALS Patient Journey into Clinical Development, Care Delivery, and Advocacy: A Research Advisory Panel <i>Session Lead: Michael Robinson, MD (ALS Research Ambassador)</i> <i>William Benjamin Nowell, PhD, MSW (Regeneron)</i> <i>John Driskell Hopkins (Hop On A Cure)</i> <i>Christina Fournier, MD (Emory University)</i>	Ballroom
9:30 – 9:50a	Break SPONSORED BY 	
9:50 – 10:50a	 A Collaborative Ecosystem for Clinical Trial Innovation <i>Session Lead: Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH)</i> <i>Jinsy Andrews, MD, MSc (NYU Langone Health)</i> <i>Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at MGH)</i> <i>Robert Bowser, PhD (Barrow Neurological Institute)</i> <i>Erin Fleming (ProJenX)</i> <i>Leigh Hochberg, MD, PhD (Mass General)</i>	Ballroom
10:50 – 11:50a	 Stats Corner: NFL — The Biomarker That Rocks Every Role <i>Session Lead: Lori Chibnik, PhD (MGH Biostatistics)</i> <i>Eric Macklin, PhD (MGH Biostatistics)</i> <i>Giorgio Paulon, PhD (Berry Consultants)</i> <i>Peng Sun, PhD (Biogen)</i> <i>Jianing Wang, PhD (MGH Biostatistics)</i>	Ballroom
11:50 – 1:10p	Lunch SPONSORED BY 	(Opal Sands) Event Lawn

2026 ANNUAL NEALS MEETING AGENDA*

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DAY TWO (CONTINUED) THURSDAY, OCTOBER 29, 2026

All meetings in Opal Sol unless otherwise noted.

12:05 – 1:05p	ARMOR Study Meeting	Equinox I
12:05 – 1:05p	Clene NIH - Expanded Access Program Meeting	TBD
12:05 – 1:05p	NEALS Biorepository Informational Session <i>Moderators: James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at MGH)</i> <i>Ikjae Lee, MD (Columbia University)</i> <i>Nadine Bakkar, PhD (Barrow Neurological Institute)</i> <i>Katie Irwin, MD, PhD Student (Johns Hopkins University)</i> <i>Hande Ozdinler, PhD (Northwestern University Feinberg School of Medicine)</i> <i>Ghazaleh Sadri-Vakili, PhD, MS (Mass General)</i>	Equinox II & III
12:05 – 1:05p	Networking Lunch: Multidisciplinary Clinics: Funding Challenges and Strategic Solutions (NEALS Members)	(Opal Sands) Sand Salons; Lunch on Mezzanine
12:05 – 1:05p	Networking Lunch: Mentorship and Career Growth with Senior Researchers (NEALS Members)	(Opal Sands) Sea Salon; Lunch on Mezzanine
1:15 – 1:30p	 Co-Chair Announcement <i>Jinsy Andrews, MD, MSc (NYU Langone Medical)</i> <i>Nicholas Maragakis, MD (Jons Hopkins)</i>	Ballroom
1:30 – 3:30p	 Science Symposium: AI in ALS: From Bench Insights to Bedside Impact SPONSORED BY  <i>Session Leads: Science Advisory Board Co-Chairs</i> <i>Sami Barmada, MD, PhD (University of Michigan)</i> <i>Timothy Miller, MD, PhD (Washington University ALS Center)</i> <i>Jeffrey Rothstein, MD, PhD (Johns Hopkins University)</i>	Ballroom
1:55p	<i>Presentations:</i> Decoding ALS with AI, From Neuron to Brain Bank: Phenotypic Signatures, Mechanism, and TDP-43 Pathology at Scale <i>Steve Finkbeiner, MD, PhD (Gladstone Institutes)</i>	
2:20p	Connecting the Clues in ALS: An Investigation Across Datasets <i>Juro Gottweis (Independent Researcher)</i> <i>Luka Rimanic, PhD (Google)</i>	
2:45p	Early Identification of ALS Using Advanced Machine Learning and Real-World Data <i>(Virtual) Vahid Esmaeili, PhD (Volvo Global)</i> <i>(Virtual) Christophe Lo Bianco, PhD (Novartis)</i>	

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DAY TWO (CONTINUED) THURSDAY, OCTOBER 29, 2026

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Scientific Symposium: AI in ALS: From Bench Insights to Bedside **Ballroom**

Impact (continued)

Session Leads: Science Advisory Board Co-Chairs

Sami Barmada, MD, PhD (University of Michigan)

Timothy Miller, MD, PhD (Washington University ALS Center)

Jeffrey Rothstein, MD, PhD (Johns Hopkins University)

3:10p

Presentations:

SAVA AI: A Collaborative Platform Connecting Clinicians, Sites, Sponsors, and Patients to Enable Real-Time Clinical Trial Matching

Natalia Luchkina, PhD (EverythingALS) and Christian Rubio, MBA (EverythingALS)

3:20p

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)

3:30 – 3:50p

Break

3:50 – 4:50p

Hot Topic Session: Causal Inference in ALS: What are the Data for Environmental Exposures **Ballroom**

Session Lead: Jonathan Glass, MD (Emory University)

Stephen Goutman, MD, MS, FAAN (University of Michigan)

Björn E. Oskarsson, MD (Mayo Clinic)

Marc Weisskopf, PhD, ScD (Harvard T.H. Chan School of Public Health)

4:50 – 6:30p

Poster Session 2

(Opal Sands) Ballroom

6:30 – 6:45p

Group Photo

(Opal Sands) Lobby

6:45 – 9:00p

Annual Beachside Dinner

SPONSORED BY



(Opal Sands) Pool Event Lawn

(End of day 2)

2025 ANNUAL NEALS MEETING AGENDA*

*Agenda subject to change

DAY THREE FRIDAY, OCTOBER 30, 2026

All meetings in Opal Sol
unless otherwise noted.

7:00a – 12:30p	Registration	Ballroom Foyer
7:00 – 8:30a	Breakfast	Ballroom Foyer
7:15 – 8:15a	FUNCTION ALS Study Meeting	Equinox II
7:15 – 8:15a	LINK Study Meeting	Equinox III
8:30 – 9:30a	 Lessons from Novel Therapies: Beyond ALS <i>Session Lead: Nicholas Maragakis, MD (Johns Hopkins University)</i> Title TBD <i>Kevin Flanigan, MD (Ohio State University)</i> Evolving treatments for SMA: Insights for ALS <i>Charlotte Sumner, MD (Johns Hopkins University)</i>	Ballroom
9:30 – 10:15a	 The Power of Partnership: Updates from the NEALS ALS Organizations Forum <i>Session Lead: Jinsy Andrews, MD, MSc (NYU Langone Medical)</i> <i>Kuldip Dave, PhD (The ALS Association)</i> <i>Kristen Cocoman (ALS United)</i> <i>Terry Heiman-Patterson, MD (ALS Hope Foundation)</i>	Ballroom
10:15 – 10:30a	Break	
10:30 – 10:50a	 Awards <i>Session Lead: Jinsy Andrews, MD, MSc (NYU Langone Medical)</i> The Gupta Family Endowed Prize for Innovation in ALS Care <i>Presented by: Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at MGH)</i> Drs. Ayeez and Shelena Lalji & Family Student Scholar Award for Repair and Regenerative Mechanisms in ALS <i>Presented by: Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at MGH)</i>	Ballroom

DAY THREE FRIDAY, OCTOBER 30, 2026

All meetings in Opal Sol
unless otherwise noted.

10:50 – 11:05a



Abstract Awards

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

Ballroom

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)

11:05 – 12:50p



Platform Presentations

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

Ballroom

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)

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 MGH)

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)

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)

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)

Presentation 6

Audience Q&A

2026 ANNUAL

NEALS MEETING AGENDA*

**Agenda subject to change*

DAY THREE

(CONTINUED)

FRIDAY, OCTOBER 30, 2026

*All meetings in Opal Sol
unless otherwise noted.*

12:50 – 2:00p	Lunch	Ballroom Foyer
1:10 – 2:10p	ALS Organizations Forum (Closed Session)	Equinox II & III

(End of day 3)