

2025 ANNUAL NEALS MEETING AGENDA*

*Agenda subject to change



Available to virtual attendees

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)

DAY ONE WEDNESDAY, OCTOBER 8, 2025

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

Amylyx A114-001 LUMINA Study Meeting

Equinox I

8:30 – 10:30a



Opening Session: NEALS at 30: A Legacy of Impact

Ballroom

Session Leads: NEALS Co-Chairs

Jinsy Andrews, MD, MSc (New York University)

James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at MGH)

Speakers:

Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at MGH)

Jeremy Shefner, MD, PhD (Barrow Neurological Institute)

Presentation:

ASO Therapies for ALS

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

10:30 – 10:50a

Break

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Healey & AMG Center

Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital

10:50 – 12:20p



NEALS Affiliated Trial Session

Ballroom

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

11:00a

Presentations:

Healey ALS MyMatch Program Update

Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at MGH)

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DAY ONE (CONTINUED) WEDNESDAY, OCTOBER 8, 2025

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10:50a – 12:20p	 NEALS Affiliated Trial Session (continued) <i>Session Lead: Jinsy Andrews, MD, MSc (New York University)</i>	Ballroom
11:15a	EPISOD1 study update on AMT-162 Gene Therapy for Individuals with SOD1 associated ALS <i>George Apostol, MD (uniQure)</i>	
11:30a	Healey ALS Platform Trial – Adaptations and Next Regimens <i>Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at MGH)</i>	
11:45a	LUMINA Study Update <i>Lauren Kett, MD, PhD (Amylyx Pharmaceuticals)</i> <i>Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH)</i>	
12:00p	ALL ALS Study Update <i>James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at MGH)</i> <i>Robert Bowser, PhD (Barrow Neurological Institute)</i>	
12:20 – 1:45p	Lunch	(Opal Sands) Pool Event Lawn
12:45 – 1:45p	Clene NIH - expanded access program meeting	Equinox I
12:45 – 1:45p	ALL ALS Meeting	Equinox II & III
12:45 – 3:15p	 Feeding Tubes and ALS: Multidisciplinary Practical Considerations Workshop Breakout	(Opal Sands) Sea Salons (Opal Sands) Sand Salons
1:10 – 2:10p	NEALS Biorepository Informational Session	(Opal Sands) Beach Salons
2:00 – 3:00p	LTX-002-101 NeurALS Study Update	Equinox I
2:00 – 3:10p	 Lighting Science Talks <i>Session Leads: Science Advisory Board Co-Chairs</i> <i>Timothy Miller, MD, PhD (Washington University ALS Center) and</i> <i>Jeffrey Rothstein, MD, PhD (Johns Hopkins University)</i>	Ballroom
	Science Presentations: 3-Minute Talks Chloroviruses and ALS <i>Gary Pattee, MD (Neurology Associates, P.C.)</i>	
	Presymptomatic gene expression changes in motor neurons in a large animal model of motor neuron degeneration <i>Stephen Kolb, MD, PhD (Ohio State University)</i>	

DAY ONE (CONTINUED) WEDNESDAY, OCTOBER 8, 2025

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2:00 – 3:10p



Lighting Science Talks (continued)

Ballroom

Session Leads: Science Advisory Board Co-Chairs

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

Jeffrey Rothstein, MD, PhD (Johns Hopkins University)

2:05p

Science Presentations: 3-Minute Talks (continued)

Phenotypic Drug Screening in Human iPSC-Derived Neurons Identifies Modifiers of Dipeptide Repeat-Induced Toxicity and TDP-43 Pathology in Amyotrophic Lateral Sclerosis/Frontotemporal Dementia

Christine Marques, PhD (Massachusetts General Hospital and Harvard Medical School)

ACE-2223, a novel oral small molecule for non genetically modified ALS, treats both gain- and loss-of-function TDP-43 pathology, extends survival and decreases NfL in vivo and has a favorable preclinical safety profile
Katie Planey, PhD, MBA (Acelot)

Talineuren (TLN): A GM1-Loaded Nanoliposomal Therapeutic Targeting Mitochondrial Dysfunction and Proteostasis in ALS

Smita Saxena, PhD (University of Missouri)

ALS-associated circular RNA hsa_circ_0000119 forms a ribonucleoprotein complex enriched with proteins central to ALS pathophysiology

Kate Smith, MD-PhD Trainee (The University of Alabama at Birmingham)

Audience Q&A

2:40p

Clinical Presentations: 3-Minute Talks

FUNcTION ALS: A Phase 1/2 trial evaluating the safety, tolerability, pharmacokinetics, pharmacodynamics and exploratory efficacy of TRCN-1023, an antisense oligonucleotide restoring UNC13A, in adult persons with Amyotrophic Lateral Sclerosis

Irina Antonijevic, MD, PhD (Trace Neuroscience)

Characterizing the CSF Biomarker Signature of Calpain-2 Activity in ALS and the Biomarker Impact of Calpain-2 Inhibition in a Preclinical Model of ALS

Robert Bowser, PhD (Barrow Neurological Institute)

Impact of Intrathecal delivery of INS1202 AAV9-SOD1-shRNA on hallmarks of neurodegeneration in a murine disease model of ALS and a patient-derived in vitro model

Laura Ferraiuolo, PhD (Insmmed Incorporated)

The Effects of Glucagon-Like Peptide-1 Receptor Agonists Medications on Amyotrophic Lateral Sclerosis

Kiran Kumari, MD (Vandalia Health)

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2:00 – 3:05p



Lighting Science Talks (continued)

*Session Leads: Science Advisory Board Co-Chairs
Timothy Miller, MD, PhD (Washington University ALS Center) and
Jeffrey Rothstein, MD, PhD (Johns Hopkins University)*

Ballroom

Science Presentations: 3-Minute Talks (continued)

Multicenter imaging study in ALS to demonstrate scalability and clinical trial readiness of multimodal MRI: Preliminary data
Pramod Pisharady, PhD (University of Minnesota)

Audience Q&A

3:30 – 4:30p



Industry Roundtable

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

*Asa Abeliovich, MD PhD (Leal Therapeutics)
Irina Antonijevic, MD, PhD (Trace Neuroscience)
Ram Miller, MDCM, MS, FRCPC (Novartis)
Shilpa Sambashivan, PhD (Nura Bio)
Bernd-Jan Sanson, MD, PhD (Prilenia)*

Ballroom

Audience Q&A

4:30 – 5:00p

Global Research & Outreach Working Group (GROW) Meeting

Estrella Hospitality Suite

4:30 – 6:30p

Poster Session 1

(Opal Sands) Ballroom

(End of day 1)

2025 ANNUAL NEALS MEETING AGENDA*

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DAY TWO

THURSDAY, OCTOBER 9, 2025

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	Prilenia Study Meeting (Closed Session)	Equinox I
7:15 – 8:15a	 Learnings from EAPs in ALS <i>Session Leads: Suma Babu, MBBS, MPH (Sean M. Healey & AMG Center for ALS at MGH) and Björn Oskarsson, MD, FAAN (Mayo Clinic)</i> Tofersen EAP Update <i>Robert Bucelli, MD, PhD (Washington University ALS Center)</i> Role of EAPs in ASO development for genetic ALS <i>Matthew Harms, MD (Columbia University)</i> Trehalose EAP Update <i>Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH)</i> Ibutilast EAP Update <i>Björn Oskarsson, MD, FAAN (Mayo Clinic)</i>	Equinox II & III
8:30 – 9:30a	 Partners in Discovery: Research Ambassadors Driving Change <i>Session Lead: Richard Bedlack, MD, PhD (Duke ALS Clinic)</i> <i>Terry Heiman-Patterson, MD (Temple University)</i> <i>Andrea Lytle Peet (ALS Research Ambassador)</i> <i>John Peffer (ALS Research Ambassador)</i> <i>Jean Swidler (ALS Research Ambassador)</i> Audience Q&A	Ballroom
9:35 – 9:45a	 ALS Better Care Act Update <i>Kelly Gwathmey, MD (Virginia Commonwealth University), Terry Heiman-Patterson, MD (Temple University), and Sabrina Paganoni, MD, PhD (Sean M. Healey & AMG Center for ALS at MGH)</i>	Ballroom
9:50 – 10:00a	 Acknowledgement of Outgoing Executive Committee Members <i>Session Lead: James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at MGH)</i>	Ballroom
10:00 – 10:20a	Break SPONSORED BY 	

2025 ANNUAL NEALS MEETING AGENDA*

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DAY TWO (CONTINUED) THURSDAY, OCTOBER 9, 2025

All meetings in Opal Sol unless otherwise noted.

10:20a – 12:20p



Scientific Symposium: Innovative Approaches and Future Directions for Treating Sporadic ALS (continued)

*Session Leads: Science Advisory Board Co-Chairs
Timothy Miller, MD, PhD (Washington University ALS Center) and
Jeffrey Rothstein, MD, PhD (Johns Hopkins University)
introducing Project Genesis*

Ballroom

Presentations:

All ALS is genetic – is this true and does it matter?
Ammar Al-Chalabi, PhD (King's College London)

Sporadic ALS iPSNs to understand disease heterogeneity and mechanistic underpinnings
Alyssa Coyne, PhD (Johns Hopkins University)

Exploring the Genetic Landscape of Nucleoporin Variants in ALS
Julia Kaye, PhD (Gladstone Institutes)

TDP-43 loss induces cryptically-spliced proteins in ALS
Sahba Seddighi, MD-PhD Candidate (Johns Hopkins School of Medicine)

Proteomics identifies a panel of plasma proteins that can be used as a diagnostic biomarker of ALS
Bryan Traynor, MD, PhD (National Institute on Aging)

12:20 – 1:45p

Lunch

(Opal Sands) Pool Event Lawn

12:45 – 2:00p

HEALEY ALS Platform Trial Study Meeting

(Opal Sands) Sea Salons

2:05 – 2:10p



Synchron BCI: Restoring Digital Independence in ALS

Omid Forouzan, PhD (Synchron)

Ballroom

2:15 – 3:15p



Hot Topic Session: Gene Therapies, now and the Future

Session Lead: Jonathan Glass, MD (Emory University)

Ballroom

*Stanley Crooke, MD, PhD (n-Lorem)
Robert Brown, MD, DPhil (University of Massachusetts Medical School)
Rebecca Ahrens-Nicklas, MD, PhD (Children's Hospital of Philadelphia)*

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DAY TWO (CONTINUED) THURSDAY, OCTOBER 9, 2025

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3:20 – 4:20p



Stats Corner: Small Trials, Big Decisions in Early Phase ALS Research

Session Lead: Lori Chibnik, PhD (MGH Biostatistics)

Presentations:

Eric Macklin, PhD (MGH Biostatistics)

Melanie Quintana, PhD (Berry Consultants)

Peng Sun, PhD (Biogen)

Ballroom

4:20 – 6:20p

Poster Session 2

(Opal Sands) Ballroom

6:20 – 6:40p

Group Photo

(Opal Sands) Lobby

6:40 – 9:00p

Annual Beachside Dinner

SPONSORED BY



**(Opal Sands) Pool Event
Lawn**

(End of day 2)

2025 ANNUAL NEALS MEETING AGENDA*

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DAY THREE

FRIDAY, OCTOBER 10, 2025

All meetings in Opal Sol
unless otherwise noted.

7:00a – 12:30p	Registration	Ballroom Foyer
7:00 – 8:30a	Breakfast SPONSORED BY 	Ballroom Foyer
7:15 – 8:15a	Healey ALS MyMatch Study Meeting	Equinox II
7:15 – 8:15a	FUNCTION ALS Clinical Team Meeting	Equinox III
8:30 – 9:00a	 Uniting for Impact: Updates from the NEALS ALS Organizations Forum <i>Session Lead: Jinsy Andrews, MD, MSc (New York University)</i> <i>Kuldip Dave, PhD (The ALS Association)</i> <i>Jerry Dawson (ALS United)</i> <i>Julie Balasalle, MSW (I AM ALS)</i>	Ballroom
9:05 – 10:15a	 Harnessing Genetics to Understand and Treat ALS <i>Session Lead: Jinsy Andrews, MD, MSc (New York University)</i>	Ballroom
9:05a	<i>Updates on developing CRISPR gene therapy for C9orf72 FTD/ALS</i> <i>Claire Clelland, MD, PhD (University of California San Francisco)</i> Audience Q&A	
10:00a	Mutant-Specific Knockdown for the Treatment of Neurodegenerative Diseases <i>John Landers, PhD (UMass Chan Medical School)</i> Audience Q&A	
10:15 – 10:30a	Break SPONSORED BY 	
10:30 – 10:50a	 Awards <i>Session Lead: Jinsy Andrews, MD, MSc (New York University)</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 10, 2025

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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

AAV-mediated gene augmentation therapy for NEK1-ALS

Katharina Meijboom, PhD (University of Massachusetts Chan Medical School)

Final Results from the Phase 3 VALOR Trial and Open-Label Extension
Evaluating Efficacy and Safety of Tofersen in Adults with SOD1-ALS

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

Progressive Upper Motor Neuron Degeneration and Neuroinflammation
in TAR4/4 Mice: A New Model for ALS and ALS/FTD

Emel Ulupinar, MD, PhD (Northwestern University)

Autologous Hybrid TREG/Th2 T Stem Cell Therapy (RAPA-501) is
Feasible and Safe in Advanced-Stage ALS Patients With Severe
Respiratory Insufficiency

James Berry, MD, MPH (Sean M. Healey & AMG Center for ALS at MGH)

Ex-Vivo Validation of a Fluorescent Clinical Retinal Tracer to Detect
TDP-43 in ALS patients With Applications in FTD and LATE

Alex Huang, MD, PhD (Amydis)

Phase 2b PARADIGM Results Demonstrate Safety, Clinical Signals,
and Biomarker Engagement of PrimeC in ALS, Supporting
Advancement to Phase 3

Merit Cudkowicz, MD, MSc (Sean M. Healey & AMG Center for ALS at MGH)

Audience Q&A

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NEALS MEETING AGENDA*

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DAY THREE

(CONTINUED)

FRIDAY, OCTOBER 10, 2025

*All meetings in Opal Sol
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12:50 – 2:00p	Lunch	Ballroom Foyer
1:10 – 2:10p	COYA 302 Phase 2 / ALSTARS Trial Study Meeting	Equinox I
1:10 – 2:10p	ALS Organizations Forum (Closed Session)	Equinox II & III

(End of day 3)