

# 2024 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 & Sub-Committee Meetings

*(open to NEALS members)*



NEALS Affiliated Trial: Study

Investigator Meetings

*(limited to site investigators & study teams)*

NEALS Member Training Sessions

*(open to NEALS members)*



Available to virtual attendees

## NEALS MEMBER ONLY DAY MONDAY, OCTOBER 21, 2024

8:00a – 4:00p	<b>Registration (Breakfast on own)</b>	<b>Opal Ballroom Foyer</b>
9:00 – 12:00p	Respiratory Care & Ventilation Committee Workshop	Opal Sand
11:00 – 12:00p	Research Coordinator Committee Mtg	Sand Dollar
11:00 – 12:00p	Upper Motor Neuron Committee Mtg	Sand Dune
<b>11:30 – 1:00p</b>	<b>Lunch</b>	<b>Opal Ballroom Foyer</b>
12:00 – 2:30p	NEALS Member Training: Site Management	Opal Sea
1:00 – 2:00p	Palliative Committee Mtg	Sand Dune
2:00 – 3:00p	Advance Practice Provider Committee Mtg	Sunset Salon
2:00 – 3:00p	fALS/FTD Committee Mtg	Beach Salons
2:00 – 3:00p	Physical Therapy/Occupational Therapy Committee Mtg	Sand Dune
3:00 – 4:00p	Nutrition Committee Mtg	Sand Dune
3:00 – 7:30p	NEALS Member Training: Outcome Measures	Opal Sea
3:00 – 4:00p	NEALS Information & Community Education (NICE) Leadership Working Group Mtg <i>(Closed Meeting)</i>	Sunset Salon
4:00 – 5:00p	Bulbar Committee Mtg	Beach Salons

*(End of NEALS Member-Only Day)*

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### DAY ONE

TUESDAY, OCTOBER 22, 2024



Available to virtual attendees

7:30a – 4:00p	<b>Registration</b>	<b>Opal Ballroom Foyer</b>
7:00 – 8:00a	Clene Nanomedicine EAP Investigators Mtg	Sunset Salon
7:30 – 8:45a	<b>Breakfast</b>	<b>Opal Ballroom Foyer</b>
7:45 – 8:30a	 CDC Informational Session: How the National ALS Registry can Support your Research	Opal Ballroom
8:30 – 10:00a	 <b>Opening Session: NEALS Updates, Progress, and Partnerships in ALS Research</b> <i>NEALS Co-Chairs</i> <i>Jinsy Andrews, MD, MSc (Columbia University)</i> <i>James Berry, MD, MPH (Healey &amp; AMG Center for ALS)</i>	<b>Opal Ballroom</b>
8:50a	NINDS's Investment in ALS Research <i>Walter Koroshetz, MD, MSc (NINDS)</i>	
9:00a	A Collaborative Approach to Advance ALS Research: The Accelerating Medicines Partnership (AMP) ALS <i>Shubhangi Lal, MS, MBA (FNIH)</i>	
9:10a	Updates on Critical Path for Rare Neurodegenerative Diseases (CP-RND) <i>Collin Hovinga, PharmD, MS, FCCP (C-Path)</i>	
9:20a	FDA update on Act for ALS Activities <i>Jacqueline Corrigan-Curray, MD, JD (Food &amp; Drug Administration)</i>	
9:30a	ALS Research and Care Roadmap for the Next Decade: Insights into the NASEM Report on ALS <i>Suma Babu, MBBS, MPH (Healey &amp; AMG Center for ALS)</i> <i>Rita Sattler, MSc, PhD (Barrow Neurological Institute)</i>	
9:50a	Q&A	





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**DAY ONE**

(CONTINUED)

**TUESDAY, OCTOBER 22, 2024**

10:00a – 10:15a	<b>Break</b>	
10:15 – 10:30a	 <b>Member Initiative Spotlight: Respiratory Care for ALS</b> <i>John Hansen-Flaschen, MD (Penn Medicine)</i>	<b>Opal Ballroom</b>
10:30 – 11:35a	 <b>NEALS Affiliated Trial Session</b>	<b>Opal Ballroom</b>
10:30a	Results and Learnings From the Phase 3 Trial Evaluating Sodium Phenylbutyrate and Taurursodiol in Amyotrophic Lateral Sclerosis (PHOENIX) <i>Sabrina Paganoni, MD, PhD (Healey &amp; AMG Center for ALS)</i> <i>Leonard van den Berg, MD, PhD (University Medical Center Utrecht)</i>	
10:45a	HIMALAYA trial: Double-blind, placebo-controlled trial of RIPK1 inhibitor (SAR443820) in ALS <i>Nazem Atassi, MD, MBA (Sanofi)</i> <i>Merit Cudkowicz, MD, MSc (Healey &amp; AMG Center for ALS)</i>	
10:55a	ALL ALS: Year 1 Progress and Future Enrollment Goals <i>James Berry, MD, MPH (Healey &amp; AMG Center for ALS)</i> <i>Robert Bowser, PhD (Barrow Neurological Institute)</i>	
11:05a	HEALEY Platform Trial updates what's new for 2025 <i>Merit Cudkowicz, MD, MSc (Healey &amp; AMG Center for ALS)</i>	
11:15a	[TALK TITLE NEEDED] <i>Andrew Satlin, MD (Transposon Therapeutics)</i>	
11:25a	Pioneering Progress: Exciting Updates from NEALS-Affiliated Trials <i>Jinsy Andrews, MD, MSc (NEALS Co-Chair; Columbia University)</i>	
11:35a	Q&A	
11:40 – 12:15	 <b>Stats Corner</b> <i>Christopher Coffey, PhD (University of Iowa)</i> <i>Eric Macklin, PhD (Healey &amp; AMG Center for ALS)</i> <i>Melanie Quintana, PhD (Berry Consultants)</i>	<b>Opal Ballroom</b>
Lunch	<b>Lunch</b>	<b>Pool Lawn</b>
12:15 – 1:15p	 <b>NEALS Research Ambassador Panel: Through the Looking Glass</b> <i>Allison Bulat (NEALS; Healey &amp; AMG Center for ALS)</i> <i>Mandi Bailey (ALS Research Ambassador)</i> <i>Cassandra Haddad (ALS Research Ambassador)</i> <i>Dave Shulman (ALS Research Ambassador)</i>	<b>Opal Ballroom</b>



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**DAY ONE**

**(CONTINUED)**

**TUESDAY, OCTOBER 22, 2024**

2:20p – 2:30p	Break	
2:30 – 3:30p	 <b>Hot Topic Session: ALS Predictive Models: How can they be used for Clinical Trials?</b> <i>Moderator: Jonathan D. Glass, MD (Emory University)</i>  <i>David Walk, MD, FAAN (University of Minnesota)</i> <i>Leonard van den Berg, MD, PhD (University Medical Center Utrecht)</i> <i>Melanie Quintana, PhD (Berry Consultants)</i> <i>David Ennist, PhD, MBA (Origent Data Sciences, Inc)</i>	<b>Opal Ballroom</b>
	<small>HOT TOPIC IN ALS SCIENCE SESSION SPONSORED BY</small> 	
3:30 – 4:30p	Big Data Committee Mtg	Opal Sun
3:30 – 4:00p	Pridopidine EAP2 Study Mtg	Beach Salons
4:00 – 4:30p	RAPA EAP Study Mtg	Beach Salons
4:30 – 6:30p	Poster Session 1	Sea Salon & Sand Salon

*(End of day 1)*

# 2024 ANNUAL NEALS MEETING AGENDA\*

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## DAY TWO

### WEDNESDAY, OCTOBER 23, 2024

7:30a – 4:00p	<b>Registration</b>	<b>Opal Ballroom Foyer</b>
7:45 – 9:15a	<b>Breakfast</b>	<b>Opal Ballroom Foyer</b>
8:00 – 9:00a	REFINE Study Mtg	Sunset Salon
8:00 – 9:00a	Global Site Mtg	Beach Salons
9:00 – 9:10a	 <b>NEALS 30th Anniversary Bike for ALS Initiative</b> <i>Jeremy Shefner, MD, PhD (Barrow Neurological Institute)</i>	<b>Opal Ballroom</b>
9:10 – 9:40a	 <b>Member Committee Spotlight: Updates from the Physical Therapist/Occupational Therapist Committee, Nutrition Committee, Respiratory Care and Ventilation Committee, and Palliative Committee</b> <i>Claire MacAdam PT, NCS (Massachusetts General Hospital)</i> <i>Michele Weaver PT, DPT (Corewell Health)</i> <i>Ambereen Mehta, MD, MPH, FAAHPM (Johns Hopkins)</i> <i>Stephanie Dobak, MS, RD, LDN, CNSC (Jefferson Weinberg ALS Center)</i> <i>Eufrosina Young, MD (SUNY Upstate Medical University)</i>	<b>Opal Ballroom</b>
9:40 – 11:30a	 <b>Science Symposium: AI is NOW: Bringing AI into ALS Research</b> <i>Moderators: Science Advisory Board Co-Chairs</i> <i>Jeffrey Rothstein, MD, PhD (Johns Hopkins University)</i> <i>Timothy Miller, MD, PhD (Washington University in St. Louis)</i>	<b>Opal Ballroom</b>
10:00a	The Genetics of ALS Reveals Potential Novel Therapeutic Targets <i>Michael Snyder, PhD (Stanford University)</i>	
10:25a	What is AI and how can it help us find causes and treatments for ALS? <i>Steven Finkbeiner, MD, PhD (University of California, San Francisco)</i>	
11:00a	Towards Better Understanding of ALS using a Multi-Marker Discovery Approach from a Multi-Modal Database (ALS4M) <i>Xing Song, PhD (University of Missouri)</i>	
11:15a	Use of AI enabled combinatorial analysis of multi-dimensional datasets to develop genetic stratification biomarkers to predict responders to existing and candidate ALS therapies and identify repurposing candidates for clinical trials <i>Steve Gardner, PhD (PrecisionLife)</i>	
11:30 – 12:30p	<b>Lunch</b>	<b>Pool Lawn</b>

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**DAY TWO**

**(CONTINUED)**

**WEDNESDAY, OCTOBER 23, 2024**

12:00p – 1:00p	NEALS Member Training: Expanded Access, Expanding Hope	Opal Sand
1:10 – 2:10p	 <b>Industry Roundtable</b> <i>Moderator: Nicholas Maragakis, MD (Johns Hopkins)</i>  <i>Lauren Kett, MD, PhD (Amylyx Pharmaceuticals)</i> <i>Simrit Parmar, MD (Cellenkos)</i> <i>Aaron Tansy, MD (PTC Therapeutics)</i> <i>Daniel Fowler, MD (Rapa Therapeutics)</i>	<b>Opal Ballroom</b>
2:15 – 4:15p	HEALEY Platform Trial Mtg	Opal Sand & Sea
2:45 – 3:45p	Biorepository Informational Session	Opal Ballroom
3:00 – 4:00p	FUSION Clinical Trial in FUS-ALS Study Mtg	Sunset Salon
4:00 – 6:00p	<b>Poster Session 2</b>	<b>Sea Salon &amp; Sand Salon</b>
6:05 – 6:20p	<b>Group Photo</b>	<b>Lobby</b>
6:20 – 7:00p	Abstract Review Committee Mtg ( <i>Closed Meeting</i> )	Sunset Salon
6:30 – 9:00p	<b>BBQ Dinner</b>	<b>Pool Lawn</b>

*(End of day 2)*

# 2024 ANNUAL NEALS MEETING AGENDA\*

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## DAY THREE

### THURSDAY, OCTOBER 24, 2023

7:30a – 12:00p	<b>Registration</b>	<b>Opal Ballroom Foyer</b>
7:45 – 9:00a	<b>Breakfast</b>	<b>Opal Ballroom Foyer</b>
8:00 – 8:55a	ALL ALS Study Mtg	Sand Salon
9:00 – 9:30a	 <b>Revolutionizing ALS Care: The Promise of Brain-Computer Interfaces</b> <i>Mariska Vansteensel, PhD (University Medical Center Utrecht)</i> <i>Leigh Hochberg, MD, PhD (Mass General Hospital)</i>	<b>Opal Ballroom</b>
9:30 – 11:55a	 <b>Awards and Abstract Platform Session</b>	<b>Opal Ballroom</b>
9:30a	The Gupta Family Endowed Prize for Innovation in ALS Care <i>Presented by: Merit Cudkowicz, MD, MSc (Sean M. Healey Center for ALS at MGH)</i>	
9:40a	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 Center for ALS at MGH)</i>	
9:50a	Big Data Committee Award <i>Presented by: Alex Sherman, (Sean M. Healey Center for ALS at MGH)</i>	
9:55a	Abstract Poster Awards <i>Presented by: Terry Heiman-Patterson, MD (Temple University)</i>	
	<b>Abstract Platform Presentations</b>	
10:05a	Boosting Clinical Trial Power in ALS with AI-Generated Digital Twins <i>Rachel Mak-McCully, PhD (Unlearn AI)</i>	
10:20a	Ataxin-2 as a target in sporadic ALS: Considerations and strategies for a path forward in light of recent trial data <i>Defne Amado, MD, PhD (University of Pennsylvania)</i>	
10:35a	Pilot Experience with Transcutaneous Carbon Dioxide Monitoring for Patients with Amyotrophic Lateral Sclerosis: A Two Center Prospective Cohort <i>Jason Ackrivo, MD, MSCE (University of Pennsylvania)</i>	
10:50a	AI for Good: Partnering with AI and Voice Bank Industry to Easily Create On-Device Personal Voice for People with ALS <i>John Costello MA, CC-SLP (Boston Children's Hospital)</i>	

**Abstract Platform Presentations**  
*(continued from previous page)*

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|----------------------|---|----------------------------|
| 11:05a               | Development of a Human iPSC-Derived “Corticospinal Tract-on-a-Chip” for ALS Research<br><i>Andriana Charalampopoulou, MD (Johns Hopkins University)</i>               |                            |
| 11:20a               | Application of 7 tesla susceptibility weighted MRI in the clinical evaluation of suspected motor neuron disease<br><i>Jaimin Shah, MD (Mayo Clinic, Jacksonville)</i> |                            |
| 11:35a               | Group Q&A   |                            |
| <b>11:55 – 1:00p</b> | <b>Lunch</b>  | <b>Opal Ballroom Foyer</b> |
| 12:00 – 1:00p        | CARDINALS Study Mtg   | Opal Ballroom              |

*(End of day 3)*