4th Annual

ALS Drug Development Summit

Accelerating Transformative Drugs to ALS & FTD Patients

May 12-14, 2025 | Boston, MA

Rethink Transformative ALS Targets, Seek Translational Biomarkers & Propel More Clinical Approvals

Your 45+ Expert Speakers Include:

Aarti Sharma
Director, Motor Neuron Disease
Regeneron

Achim Kless
Senior Director
Eli Lilly

Amanda Guise
Principal Scientist, Biomarkers & Systems Biology
Biogen

Makoto Tamura
Senior Director, NeuroDiscovery Lab
Mitsubishi Tanabe Pharma America

Robert Bowser
Chief Scientific Officer
Barrow Neurological Institute

Sabrina Paganoni
Co-Director, Neurological Clinical Research Institute
Massachusetts General Hospital

Lead Partner:

Quanterix

2025 Partners

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UNITING INNOVATION, COLLABORATION & DETERMINATION TO DRIVE BREAKTHROUGHS & TRANSFORM OUTCOMES IN ALS & FTD

With **QurAlis** and **Eli Lilly's** recent announcement to develop and commercialize their ASO to restore *UNC13A* function in ALS and FTD, Trace Neuroscience receiving $101 million in funding, new innovation in TDP43 biomarkers and target understanding, and plenty of regulatory endpoint challenges, drug developers in ALS and FTD have much to discuss. At the same time, the removal of RELYVRIO from the marker still highlights the need to rethink and develop new transformative drugs for patients in 2025.

This year, the **4th ALS Drug Development Summit**, reunites a 150+ strong community of ALS & FTD stakeholders: a rich tapestry of perspectives across discovery, biology, preclinical, translational, clinical and regulatory experts from across large pharma and biotech, as well as key stakeholders from clinicians, academics, funding and research organizations, patients and caregivers. Together we aim to learn from late stage trial failures, leverage emerging breakthroughs and drive more impactful research to advance treatments and ultimately a cure for sporadic and familial ALS.

Hear from previous speakers:

"A great summit to connect Preclinical and Clinical thought leaders on ALS and to garner recent progress for breakthrough ALS therapies."

Mansuo Lu Shannon, Chief Scientific Officer, **AskBio**

"The ALS Drug Development Summit provides a unique opportunity to disseminate knowledge and share ideas with other industry partners and regulators in order to identify the best ways to get promising new therapies to those who need them the most."

Christopher Ocampo, Senior Medical Director, **AbbVie**

Expand Therapeutic Horizons Within & Beyond TDP-43 Pathology: Explore unpublished findings on the latest targets, including *UNC13A*, *STMN2*, mitochondrial dysfunction, and Tregs, to unlock innovative therapeutic avenues for ALS and FTD in clinical and preclinical validation

Enhance Translational Confidence in Preclinical Models: Leverage advanced cocultures, organoids, and patient-derived iPSC models to better replicate sporadic ALS pathology and address donor variability

Optimize Clinical Trial Strategies: Incorporate decentralized and adaptive designs, digital biomarkers, and refined endpoints to improve trial efficiency, patient access, and regulatory alignment

Advance Biomarker Development for Earlier Detection: Utilize cutting-edge tools like NFL, TDP-43 PET tracers and plasma biomarkers and metabolic biomarkers, to stratify patients and monitor disease progression with greater precision

Collaborate for Accelerated Progress: Engage with global consortia, regulatory bodies, and patient advocacy groups to streamline data sharing, expand genetic testing, and ensure patient-centric drug development pathways

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YOUR 45+ EXPERT SPEAKERS

Aarti Sharma
Director, Motor Neuron Disease
Regeneron

Achim Kless
Senior Director
Eli Lilly

Amanda Guise
Principal Scientist, Biomarkers & Systems Biology
Biogen

Amalia Papanikolaou
Data Programme Lead
Challenge Works, Nesta

Andrew Lo
Professor
Massachusetts Institute of Technology

Anil Tarachandani
Vice President & Head of Translational Medicine
Verge Genomics

Arti Patel
Principal Scientist & Group Leader
Novartis

Asa Abeliovich
Chief Executive Officer & Founder
Leal Therapeutics

Bob Dagher
Executive Vice President & Chief Medical Officer
Brainstorm Cell Therapeutics

Christopher Bunker
Chief Business Officer
Alamar Biosciences

Daniel Fowler
Chief Medical Officer
Rapa Therapeutics

Deborah Bellina
ALS Advoacate & Caregiver to her son Matt

Ellen Guss
Scientific Programs Coordinator
Target ALS

Emma Bowden
Senior Vice President & Head of Development
QurAlis

Erin Fleming
Chief Operating Officer
ProJenX

Ernest Fraenkel
Principal Investigator, Data Analytics
Answer ALS

Ethan Ash
EVP, Business Development
Bionews

Fernando Vieira
Chief Executive Officer & Chief Scientific Officer
ALS TDI

Hariprasad Vankayalapati
Chief Scientific Officer
Biolexis Therapeutics

Ignazio Di Giovanna
VP, Scientific Affairs, Neurology
Atlatic Research Group

Isaac Klein
Chief Scientific Officer
Dewpoint Therapeutics

James Berry
Associate Neurologist
Massachusetts General Hospital

Jason Thonhoff
Head of Clinical Development
Coya Therapeutics

John Nieland
Co-Founder & Chief Scientific Officer
2N Pharma

YOUR 45+ EXPERT SPEAKERS

Leticia Tarilonte
Head of Clinical Operations
Brainstorm Cell Therapeutics

Makoto Tamura
Senior Director, NeuroDiscovery Lab
Mitsubishi Tanabe Pharma America

Marcel Van Der Brug
Chief Scientific Officer
AcuraStem

Marcella Debidda
President Patient Insights & Clinical Solutions
BioNews Clinical

Mark Roskey
Chief Scientific & Collaboration Officer
Quanterix

Martin Jacko
Chief Executive Officer
Aperture Therapeutics

Mary Kay Turner
Senior Vice President, Global Patient Advocacy & Public Affairs
Brainstorm Cell Therapeutics

Matt Mandeville
Applications & BD Manager
BrainXell

Michael Thurn
Chief Executive Officer
Neurizon

Netta Blondheim-Shraga
Senior Vice President, Research & Development
Brainstorm Cell Therapeutics

Olga Uspenskaya-Cadoz
Chief Medical Officer
VectorY Therapeutics

Philip C. Wong
Professor of Pathology
John Hopkins Medicine

Ray Kelleher
Managing Director
Cormorant Asset Management

Richard Fisher
Chief Scientific Officer
Eikonizo Therapeutics

Richard Ransohoff
Venture Partner
Third Rock Venturers

Robert Bowser
Chief Scientific Officer
Barrow Neurological Institute

Ron Hoffman
Founder & Executive Director
Compassionate Care ALS

Sabrina Paganoni
Co-Director, Neurological Clinical Research Institute
Massachusetts General Hospital

Terry Fang
Senior Director, Preclinical Science
Trace Neuroscience

Tetsuya Tanaka
Vice President, Stem Cell Biology
Elixirgen Scientific

Timothy Piser
Head of Research & Development
Samsara Therapeutics

Vern Norviel
Chief Medical Officer
Neuvivo

Wendy Hendrickson
ALS Hope Foundation

PRE-CONFERENCE WORKSHOP DAY - MAY 12, 2025

8:30 Morning Check-In: *served with coffee + light breakfast*

WORKSHOP TRACK A
DISCOVERY & PRECLINICAL

Designed for CSOs, preclinical researchers, discovery scientists, directors of biology and research, novel modality scientists, in vivo/in vitro modeling experts, and many more; connect with an intimate group of discovery and preclinical experts for a more discursive session

9:00 Workshop A: Pioneering Modalities for ALS: Unpacking the Gene Therapy Toolbox & Beyond

From gene editing and augmentation to emerging delivery systems and knockdown strategies, the session will provide a comprehensive exploration of the gene therapy landscape. Participants will discuss which technologies are best suited for targeting specific ALS mutations, the challenges of sporadic versus hereditary forms, and the broader application of current platforms. By analyzing both successes and setbacks, such as the lessons from C9orf72 and SOD1, the workshop aims to define a roadmap for optimizing genetic medicines "for ALS."

- Examine the potential of gene editing, viral and non-viral delivery, and vectorized antibodies, and their relevance in various use cases to address ALS pathologies effectively
- Strategize on targeting more complex mutations e.g C9orf72 requiring balancing gain- and loss-of-function approaches within the gene therapy framework
- Identify how current platforms and delivery technologies can overcome challenges in targeting sporadic and hereditary ALS populations
- Evaluate the potential of peripheral administration for gene therapies to explore comparable efficacy and safety
- Navigate future directions and regulatory requirements for gene therapy in ALS, exploring new modalities and technologies to enhance therapeutic impact and accessibility

Philip C. Wong, Professor of Pathology, **Johns Hopkins Medicine**

Asa Abeliovich, Chief Executive Officer & Founder, **Leal Therapeutics**

WORKSHOP TRACK B
CLINICAL & TRANSLATIONAL UTILITY

Tailor-made for medical directors, CMOs, clinical program leads, translational and clinical development heads, clinicians, and more; share expertise from diverse clinical challenges and innovation in ALS

9:00 Workshop B: Decoding Clinical Endpoints in ALS: Revolutionizing Drug Development with Emerging Biomarkers, ALS-FRS-R & Digital Endpoints

This workshop explores the ALS Functional Rating Scale-Revised (ALSFRS-R) as a cornerstone clinical endpoint, addressing its limitations and strategies to enhance its impact. We'll investigate the role of digital biomarkers, such as wearables and smartphone-based assessments, alongside other primary and secondary endpoints like respiratory function, survival, and quality of life. Attendees will gain actionable insights into building a robust, multidimensional framework for trial design, aligned with regulatory requirements, to accelerate ALS drug development and approvals.

- Critically examine the ALSFRS-R to analyze the strengths, limitations, standardization concerns and key considerations when using ALSFRS-R as a clinical endpoint, including its sensitivity to disease heterogeneity
- Evaluate the role of wearables, smartphone-based assessments, and AI-driven analysis as digital endpoints in capturing nuanced progression signals for ALS
- Integrate diverse endpoints to discuss the interplay of primary, secondary, and exploratory endpoints—such as respiratory function, survival rates, and quality-of-life measures—in building a comprehensive efficacy profile
- Streamline approvals to align endpoint selection with regulatory expectations, fostering more efficient pathways for drug approval

Fernando Vieira, Chief Executive Officer & Chief Scientific Officer, **ALS TDI**

12:00 Lunch & Networking

1:00 Workshop C: To What Extent Does TDP-43 Underlie ALS Pathology? Evaluating Novel Target Discovery & Validation to Address the Breadth of ALS Pathology

This workshop will delve into TDP-43's role as a key driver of ALS pathology and explore broader mechanisms contributing to disease heterogeneity. Attendees will critically assess TDP-43 as a therapeutic target, how well TDP-43 accounts for all ALS pathology in sporadic ALS, identify other pathological drivers such as neuroinflammation and metabolic dysfunction, and refine preclinical strategies to validate novel targets. The discussion will address the challenges of model selection, biomarker development, and the integration of TDP-43-targeted therapies into a multifaceted ALS treatment landscape.

- Evaluate TDP-43's role in ALS pathology and its interaction with non-neuronal mechanisms, including neuroinflammation, mitochondrial dysfunction, and lipid metabolism
- Explore the utility of TDP-43 biomarkers and preclinical models in the context of target validation and preclinical development of novel drug mechanisms beyond TDP-43
- Discuss the positioning of TDP-43-targeted therapies within a broader ALS treatment landscape to address disease heterogeneity
- Refine preclinical strategies for better target validation and enhanced predictability of therapeutic efficacy in humans

Aarti Sharma, Director, Motor Neuron Disease, **Regeneron**

Arti Patel, Principal Scientist, Neuroscience Program Lead, **Novartis**

1:00 Workshop D: Establishing Adaptive & Decentralized Models to Accelerate Therapeutic Discovery in ALS

This workshop will explore the transformative potential of adaptive and decentralized clinical trial designs in ALS research. By integrating platform trials, multi-arm designs, and decentralized tools, these approaches aim to enhance trial efficiency, broaden patient access, and improve the trial experience for participants. Discussions will focus on optimizing recruitment, leveraging interim data for trial modifications, and utilizing digital tools to reduce patient burden while ensuring regulatory alignment.

- Evaluate adaptive trial designs, including platform trials and response-adaptive randomization, optimize recruitment, improve data collection, and enhance trial efficiency
- Explore strategies to improve patient engagement, streamline enrollment, and diversify participation while maintaining trial integrity
- Assess decentralized trial methodologies such as remote monitoring, self-reported ALS-FRS-R, and home visits to mitigate patient burden and foster inclusivity
- Examine operational challenges and regulatory requirements for implementing these models effectively in ALS research

Bob Dagher, Executive Vice President & Chief Medical Officer, **Brainstorm Cell Therapeutics**

Leticia Tarilonte, Head of Clinical Operations, **Brainstorm Cell Therapeutics**

4:00 End of Preconference Day

CONFERENCE DAY ONE - TUESDAY, MAY 13, 2025

7:00 **Morning Check-In:** *served with coffee + light breakfast*

7:55 **Chair's Opening Remarks**

FUTURE DIRECTIONS & RESEARCH PRIORITIES: WHAT WILL THE ALS LANDSCAPE LOOK LIKE IN YEARS TO COME?

8:00 **Advancing ALS Drug Development Through the HEALEY ALS Platform Trial: Accelerating Innovation & Collaboration**

- Highlighting the design and success of the HEALEY ALS Platform Trial in streamlining drug development and improving trial efficiency through a shared infrastructure
- Discussing insights from completed and ongoing regimens, including biomarker discoveries, trial outcomes, and implications for ALS treatment strategies
- Exploring the collaborative framework of the platform, fostering partnerships between academia, industry, and patient communities to drive innovation in ALS therapeutics

Sabrina Paganoni
Co-Director, Neurological Clinical Research Institute
Massachusetts General Hospital

8:30 **Unlocking the Power of Blood-Based Biomarkers in ALS: From Discovery to Clinical Impact**

- Explore how Simoa® technology is enabling ultra-sensitive detection of biomarkers like Neurofilament light chain (NfL) to monitor ALS progression and treatment response
- Gain real-world insights into how NfL is being used in clinical trials to support patient stratification, therapeutic monitoring, and trial design
- Understand the future of personalized ALS care through the integration of fluid biomarkers into research, diagnostics, and disease management

Mark Roskey
Chief Scientific & Collaboration Officer
Quanterix

9:00 Panel Discussion: **Strategic Funding & Resource Allocation in ALS Research: Overcoming Barriers to Clinical Development**

- ALS trials are highly resource-intensive, with significant financial and logistical demands, creating a major barrier for therapies entering development
- Strategies like synthetic control arms, remote visits, and digital biomarkers can cut costs and reduce patient burden, though validation of these technologies remains a challenge
- Investors prioritize ALS programs with unique therapeutic approaches, including gene therapy and alternatives to heavily funded pathways like TDP-43, focusing on clinical differentiation and scalability
- Partnerships between startups, big pharma, and venture funds are key to addressing validation gaps and improving the efficiency of ALS clinical trial execution while prioritizing patient needs

Moderator
Sabrina Paganoni
Co-Director, Neurological Clinical Research Institute
Massachusetts General Hospital

Andrew Lo
Professor
Massachusetts Institute of Technology

Ray Kelleher
Managing Director
Cormorant Asset Management

Richard Ransohoff
Venture Partner
Third Rock Venturers

9:30 **Speed Networking**

A prime chance to make the most of in-person networking and forge new connections as new companies enter, and existing ones broaden their presence within the ALS drug development space. Designed to maximize your introduction to numerous new individuals and serve as a catalyst for ongoing discussions during the summit.

10:00 **Morning Break & Refreshments**

CONFERENCE DAY ONE - TUESDAY, MAY 13, 2025

DISCOVERY & PRECLINICAL TRACK

PUSHING THE BOUNDARIES OF ALS MODELING: ENHANCING PRECLINICAL SYSTEMS FOR PRECISION & TRANSLATIONAL SUCCESS

Chair: **Aarti Sharma,** Director, Motor Neuron Disease, **Regeneron**

10:30 Modeling ALS Pathophysiology Using Patient-Derived iPSCs for Therapeutic Insights

- Establishing patient-derived iPSC-neurons harboring TDP-43 mutations, providing a robust in vitro model to recapitulate key pathological features of ALS
- Comprehensive analyses to investigate the mechanisms of action of currently available ALS therapies, offering deeper insights into their therapeutic impact
- Identifying novel drug targets, paving the way for the development of innovative treatments for ALS

Makoto Tamura, Senior Director, NeuroDiscovery Lab, **Mitsubishi Tanabe Pharma America**

11:00 Advancing ALS Drug Discovery with iPSC-Derived Neurons & Glia

- BrainXell provides a range of human iPSC-derived neurons and glia from healthy and disease lines for use in drug discovery applications
- Differentiating ALS patient lines harboring mutations in disease-relevant genes (i.e. C9ORF72, SOD1, TDP-43) into various cells of the CNS and generating isogenic controls
- Disease modeling with mono-, co-, and triculture systems to advance personalized medicine for ALS and other neurodegenerative diseases

Matt Mandeville, Applications & BD Manager, **BrianXell**

11:10 Embracing Heterogeneity in ALS to Develop Diverse *In Vitro* Models that can Support Development of Targeted Therapeutics

- ALS patients present with significant heterogeneity in disease features, that may share related, common mechanisms
- Most ALS disease models focus on rare forms of the disease and are not genetically representative of >80% of patients with sporadic ALS
- Developing and selecting drug candidates in diverse patient cell lines is standard practice in oncology therapeutic discovery
- Development of a large panel of patient models and iPSC lines has facilitated the rapid discovery and development of UNC13A targeting antisense oligonucleotide (ASO) therapeutics

Marcel Van Der Brug, Chief Scientific Officer, **AcuraStem**

CLINICAL & TRANSLATIONAL UTILITY TRACK

TRANSLATING CUTTING EDGE BIOMARKER ADVANCEMENTS INTO CLINICAL UTILITY FOR EARLIER DIAGNOSIS, IMPROVED PREDICTIVITY & PATIENT STRATIFICATION

Chair: **Olga Uspenskaya-Cadoz,** Vice President, Clinical Development, **Eli Lilly**

10:30 Evaluating Multimodal Effects of Debamestrocel on Varying CSF Biomarker Pathways in ALS Linked to Clinical Outcomes

- Showcasing the potential multimodal mechanism of action of Debamestrocel through analysis of 45 biomarkers by two subgroups characterized by ALS-FRS-R into a panel approach, to build a clearer and more holistic picture of disease progression and Debamestrocel efficacy on the complex and heterogenous pathology within ALS
- Reviewing the clinical utility of NfL and NfH as reliable biomarkers of disease progression and treatment monitoring in ALS, while examining how longitudinal data has refined their use in predicting therapeutic outcomes
- Advancing neuroprotective strategies through biomarkers to explore the role of biomarkers like BDNF and GDNF in informing alignment with cellular and molecular repair mechanisms
- Sharing regulatory insights and next steps for clinical development: study's recommendations and recent advancements

Netta Blondheim-Shraga, Senior Vice President, Research & Development, **Brainstorm Cell Therapeutics**

11:00 Session TBC

11:30 Condensate Modulation as a Treatment Modulation for ALS & FTD

- The role of condensates in neurodegenerative disease
- Condensate modulation as a therapeutic approach for neurodegenerative disease
- Condensate modulator discovery and development for ALS and FTD

Isaac Klein, Chief Scientific Officer, **Dewpoint Therapeutics**

CONFERENCE DAY ONE - TUESDAY, MAY 13, 2025

11:40 Panel Discussion: **To What Extent are *In Vivo* Models Relevant Purely in Evaluating Toxicity & Target Engagement?**

- Highlighting the need for robust toxicity and bioavailability data showcased *in vivo* systems
- Critically evaluating the ability of animal models to predict drug efficacy in treating ALS patients, and the relevance of target engagement data *in vivo* with different modalities of treatment (including gene therapies)
- Navigating regulator perspective across FDA, EMA and more to the credence placed on animal and human cell data
- Determining to what degree *in vitro* models can provide robust efficacy data for regulators and to determine clinical efficacy, what *in vivo* data might be necessary to prove efficacy and safety

Martin Jacko, Chief Executive Officer, **Aperture Therapeutics**

"Great opportunity to learn about new trends in ALS research. Also, excellent networking opportunity."

Chief Medical Officer, **Xalud Therapeutics**

12:10 Lunch & Networking

UNLOCKING GENETIC & PROTEOMIC INSIGHTS TO VALIDATE NOVEL BIOMARKERS, TARGETS & MECHANISMS IN ALS

1:00 Identifying Protective Variants in Neurodegeneration with the UK Biobank: Expanding Insights to ALS, FTD, Spinocerebellar Ataxia & Beyond for More Effective Drug Development

- Harnessing whole exome and whole genome sequencing to identify protective/rare variants and their subsequent prioritization
- Exploring the influence of causal variants in the context of mutations in the rest of the genome (epistasis and penetrance)
- Taking learnings from Spinocerebellar Ataxia omics to further differentiate disease and stratify patient subgroups with different pathologies
- Identifying subgroups based on comorbidities and diagnosis using examples from the UK biobank

Achim Kless, Senior Director, **Eli Lilly**

1:30 Unbiased Proteomic Analysis of Clinical Trial CSF Samples to Identify Early Pharmacodynamic Response Signatures Associated with Disease-Modifying Therapies in ALS

- Identification of tofersen PD-response biomarkers in SOD1-ALS VALOR clinical trial CSF via multiplexed quantitative proteomics
- Significant modulation from baseline abundance observed for 56 proteins in tofersen-treated participants relative to placebo, including proteins with significant changes from baseline as early as 4 weeks post-treatment
- Orthogonal validation of selected biomarker candidates across independent tofersen-treated cohorts
- Utility of integrating unbiased proteomic screening with targeted validation methods to identify new predictive, prognostic, and pharmacodynamic response biomarkers in clinical trial biospecimens

Amanda Guise, Principal Scientist, Biomarkers & Systems Biology, **Biogen**

INNOVATIVE TRIAL DESIGNS & PATIENT-CENTRIC STRATEGIES: ENHANCING REGULATORY ALIGNMENT TO ACCELERATE ALS DRUG APPROVALS & IMPROVE PATIENT OUTCOMES

1:00 Learning from the Past: A Landscape Analysis of Recent Failures in ALS Drug Development to Inform Future Successes

- Overviewing key readouts of recent clinical failures to uncover pitfalls in ALS drug development to quicken the pace of research
- Examining key challenges in ALS development, including translational gaps in preclinical models, ineffective patient stratification, and trial design issues that hinder clinical success
- Highlighting the importance of biomarker-driven strategies, multi-omics integration and adaptive trial designs to address diseases heterogeneity and improved success rates

Anil Tarachandani, Vice President & Head of Translational Medicine, **Verge Genomics**

1:30 Panel Discussion: **Given Recent Failures, How Can we Tailor ALS Clinical Trials to Successfully Meet Regulatory Expectations**

- Outlining the approval path for sporadic and familial ALS: is it the same for different modalities? What is the regulatory bar for approving this?
- Harnessing innovative trial design
- Cross comparing diverging requirements for different regulatory agencies including the FDA, EMA and more
- Establishing clear, patient-relevant endpoints and leveraging biomarkers to demonstrate efficacy
- Critically identifying stumbling blocks in IND and BLA packages to circumvent late-stage failures and pitfalls in drug development

Anil Tarachandani, Vice President & Head of Translational Medicine, **Verge Genomics**

Ignazio Di Giovanna, VP, Scientific Affairs, Neurology, **Atlatic Research Group**

Olga Uspenskaya-Cadoz, Vice President, Clinical Development, **Eli Lilly**

CONFERENCE DAY ONE - TUESDAY, MAY 13, 2025

2:00 ALS Patient-derived iPSC Models for Advancing Drug Discovery

- Development of multiple neuronal & glial cell types from healthy and disease patient iPSC lines
- TDP43 mislocalization in ALS-patient iPSC derived motor neuron
- Current capabilities and potential future applications in advancing personalized medicine for ALS disease & other neurodegenerative diseases
- Demonstrating how these models improve target discovery, biomarker validation, and preclinical predictability in drug development

Tetsuya Tanaka, Vice President, Stem Cell Biology, **Elixirgen Scientific**

2:00 AI-Generated Digital Twins in ALS: Improving Confidence in Early-Stage Studies

- PRO-101 - Study Overview: a Phase 1 clinical trial evaluating prosetin—a first-in-class MAP4 kinase (MAP4K) inhibitor
- AI-Powered Digital Twins in - PRO-101 – Enhancing data robustness and interpretation in ALS studies
- Using Digital Twins as an External Control – Leveraging AI to support early-stage data analysis for more informed decision-making

Erin Fleming, Chief Operating Officer, **ProJenX**

2:30 Discovery of Protective Genetic Variants to Develop Disease Modifying Therapeutics for ALS

- Unpacking the role of genetic heterogeneity in ALS
- Shifting perspective - protective genetic variants in non-neuronal genes that impact disease progression and survival
- Development of targeted disease modifying therapeutics

Martin Jacko, Chief Executive Officer, **Aperture Therapeutics**

2:30 Clinical Translation of Hybrid Treg/Th2 (RAPA501) Cells for Therapy of ALS

- Mechanism of action of RAPA501
- Phase 1 results; manufacturing, safety and immune modulation
- Implementing 40 patient Expanded Access trial (<50% pulmonary function)
- Implementing Phase 2/3 trial (>70% pulmonary function)

Daniel Fowler, Chief Medical & Scientific Officer, **Rapa Therapeutics**

3:00 Scientific Poster Session

This is an informal session to help you connect with your peers in a relaxed atmosphere to continue forging new and beneficial relationships. With an audience of preclinical, translational, and clinical scientists eager to hear the latest advancements in ALS therapeutic development, you will have the opportunity to display a poster presenting your own work and innovations.

3:30 Afternoon Break & Refreshments

HARMONIZING CONSORTIA LEARNINGS TO TRANSFORM DRUG DEVELOPMENT IN ALS

4:00 **Target ALS: A Catalyst for Drug Discovery**

- Research We Enable: Democratize ALS research worldwide by providing investigators access to critical research tools and resources with no strings attached
- Research We Fund: Break down silos to forge academic-industry collaborations and bring together labs with cutting edge complementary expertise to accelerate ALS drug discovery through innovative funding programs
- Research We Conduct: Lower barriers for underrepresented communities and minorities to impact clinical research through global initiatives that help create one-of-a-kind comprehensive and accessible repositories of biosamples and big data to galvanize AI/ML application in ALS drug discovery

Ellen Guss
Scientific Programs Coordinator
Target ALS

4:30 **The Neuromine Data Portal & the Value of Big Data Analytics in ALS Research**

- Answer ALS to Neuromine's open-access data resources
- The power of the data
- Partnerships and workbenches on the horizon

Ernest Fraenkel
Professor of Biological Engineering, **MIT** & Principal Investigator, Data Analytics, **Answer ALS Research**

CONFERENCE DAY ONE - TUESDAY, MAY 13, 2025

5:00 **The Longitude Prize on ALS: A Groundbreaking Global Prize Harnessing the Power of AI to Drive Treatment for ALS**

- The Longitude Prize series brings together the brightest minds to solve the world's most challenging innovation problems
- The Longitude Prize on ALS, launching in June 2025, will bring together computational biologists, neurodegenerative researchers and AI-driven biotech globally to uncover novel therapeutic targets for ALS

Amalia Papanikolaou
Data Programme Lead
Challenge Works, Nesta

5:10 **ALL ALS: Establishing a National Research Consortium to Accelerate ALS Drug Discovery and Harmonize Global Data**

- ALL ALS is an NIH-funded initiative to enroll nearly all ALS patients in the U.S., collecting clinical data, biosamples, and digital health measures through site-based and remote methods
- Integrating datasets from Target ALS, Answer ALS, and AMP-ALS into a public portal to accelerate research and therapeutic development
- Harmonizing ALS research data with other neurological diseases, such as Parkinson's and FTD, to explore shared mechanisms and disease progression
- Partnering with international groups, ALL ALS aims to expand its reach, unify global research efforts, and drive biomarker discovery

James Berry
Associate Neurologist
Massachusetts General Hospital

Robert Bowser
Chief Scientific Officer
Barrow Neurological Institute

5:40 Panel Discussion: **Uniting Forces: Collaborative Initiatives Driving ALS Drug Development Through Data Sharing & Accelerated Research**

- How are these initiatives complementing each other in the ALS research space?
- What are the biggest hurdles in harmonizing and sharing data across these programs?
- How do we ensure that patient-centric approaches remain at the forefront of these efforts?
- What are the best practices for ensuring standardization and quality of collected data?
- How can international collaborations expand the scope and utility of your programs' data?
- What does success look like for collaborative consortia and research programs in the next 3–5 years?

Fernando Vieira
Chief Executive Officer &
Chief Scientific Officer
ALS Therapy Development Institute

James Berry
Associate Neurologist
Massachusetts General Hospital

Robert Bowser
Chief Scientific Officer
Barrow Neurological Institute

6:10 **Chair's Closing Remarks**

6:15 **Drinks Reception**

7:15 **End of Conference Day One**

"This event helped me connect pre-clinical developments with clinical advancements. Additionally, it provided an opportunity to learn from the community about various important aspects of drug development from different perspectives. The experience was very insightful."

Senior Scientific Director, **CPath**

CONFERENCE DAY TWO - WEDNESDAY, MAY 14, 2025

7:30 **Morning Check-In:** *served with coffee and a light breakfast*

7:55 **Chair's Opening Remarks**

INNOVATING A NEW ERA OF EARLIER DIAGNOSIS & PROGNOSIS TO ADVANCE STRATIFICATION OF HETEROGENOUS ALS POPULATIONS

8:00 **Refining Sensitivity & Specificity of TDP-43 Loss of Function Assay in Plasma for Sporadic ALS for Diagnostic & Trial Utility**

- Outlining HDGFL2 cryptic neoepitope as a potential early diagnostic marker for ALS, identifying TDP-43 splicing repression in plasma of sporadic ALS patients, upstream of aggregate formation
- Harnessing TDP-43 splicing repression to identify ALS onset upstream of aggregate pathology and earlier in disease for earlier diagnosis
- Evaluating longitudinal changes in disease progression and patient benefit to determine predictivity of drug effect
- Improving assay sensitivity to better differentiate disease and controls to ensure assay qualification and validation to clinical use standards for interpreting trial results, while improving trial design and context of use

Philip C. Wong
Professor of Pathology
Johns Hopkins Medicine

8:30 **NULISA CNS Disease Biomarker Platform**

- The only high-plex, high sensitivity platform for investigation of biomarkers of neurodegenerative diseases and treatment response
- NULISA™ combines ultra-sensitive femtogram level detection with barcode-based high-plex biomarker analysis
- The NULISA CNS Disease Panel is unique in enabling investigation of hundreds of proteins involved in neuroinflammation, vascular, and synaptic dysfunction, and proteinopathies
- NULISA offers the unique ability to translate multiplexed biomarker profiling for discoveries to single and low-plex biomarker assays run with the same assay on the same instrument
- NULISA single-, low-, and high-plex assays all run fully automated on the ARGO HT System
- NULISA CNS Disease Panel 120 and Inflammation Panel 250 are used for exploratory clinical research into therapeutic effects on neurodegenerative disease processes
- NULISA IVD and ARGO DX in development with funding from Gates Ventures and DxA

Christopher Bunker
Chief Business Officer
Alamar Biosciences

9:00 **Can we Slow Neurodegeneration in Multiple Indications without Reducing Neurofilament Light Chain?**

- Reviewing data on NFL in different neurodegenerative diseases
- Mechanisms and factors that contribute to NFL levels in biofluids
- Exploring examples of different ALS therapeutic trials where NFL was reduced with treatment response
- Does the timing of NFL reductions during trials depend upon the mechanism of action of the treatment?
- Evaluating feasibility for controlling baseline heterogeneity with NFL for more confident confirmation of efficacy

Robert Bowser
Chief Scientific Officer
Barrow Neurological Institute

CONFERENCE DAY TWO - WEDNESDAY, MAY 14, 2025

9:30 **Improving Disease Scoring Systems in ALS: Addressing Heterogeneity & Decline Rates to More Accurately Interrogate Drug Efficacy in Patient Subgroups**

- Understanding ALS heterogeneity to explore the key factors contributing to ALS diverse progression rates and how heterogeneity can be accounted for in clinical scoring systems
- Overviewing current ALS-FRS scoring systems and their limitations in both clinical trials and routine care, and why current scores may not adequately reflect all patients' experience (e.g. patients with different onset ages or rates of progression)
- Innovative approaches to scoring ALS progression including new methods to improving scoring and patient stratification, the role of biomarkers in enhancing scoring systems and the power of AI and machine learning to identify patterns of progression that are missed by traditional scores
- Exploring implications of improved scoring on ALS trial design (adjusting for disease onset and progression speed), regulatory acceptance and approval processes

James Berry
Associate Neurologist
Massachusetts General Hospital

10:00 **Morning Break & Refreshments**

DISCOVERY & PRECLINICAL TRACK

PRECLINICAL VALIDATION FOR ALS TO HERALD A NEW ERA OF SAFER & MORE EFFECTIVE DRUGS TO THE CLINIC

Chair: **Aarti Sharma,** Director, Motor Neuron Disease, **Regeneron**

10:30 A Novel Activator of Autophagy Rescues Autophagy Dysfunction & Reduces C9- & TDP43-ALS Pathology in iPSC-derived Motor Neurons & *In Vivo*

- Uncovering potent small molecule activators of autophagy as a novel approach to treating C9- and TDP43-ALS
- Revealing preclinical proof of concept in patient iPSCs and mouse models
- Discussing the path to clinical development

Timothy Piser, Head of Research & Development, **Samsara Therapeutics**

11:00 Development of EKZ-102, a CNS-Penetrant Small Molecule HDAC6 Inhibitor as a Potential First-in-class Disease-Modifying Therapeutic for ALS

- Eikonizo is developing highly selective, potent, orally bioavailable, CNS-penetrant, first-in-class small molecule HDAC6 inhibitors for neurodegeneration
- Development candidate, EKZ-102, is a potentially disease-modifying therapeutic for both sporadic and familial ALS designed to correct broad proteostasis and intracellular transport defects to protect neurons and preserve function
- EKZ-102 IND-enabling studies are underway with planned clinical start in late 2025

Richard Fisher, Chief Scientific Officer, **Eikonizo Therapeutics**

CLINICAL & TRANSLATIONAL UTILITY TRACK

GENETICALLY VALIDATED TARGETS & PRECISION THERAPEUTICS: ADVANCING ALS TREATMENT WITH UNC13A RESTORATION & STMN2 SPLICING TECHNOLOGIES IN THE CLINIC

Chair: **Olga Uspenskaya-Cadoz,** Vice President, Clinical Development, **Eli Lilly**

10:30 UNC13A Restoration as a Novel Genetically Validated Approach to ALS

- Exploring Genetics in ALS: across rare Familial Forms and sporadic population
- Target validation, mechanisms and biology of UNC13A
- Discovery and development of medicines for UNC13A

Terry Fang, Senior Director, Preclinical Science, **Trace Neuroscience**

11:00 Recovering Misplacing Errors with QRL-201 & QRL-101 to Restore Function & Reduce Disease Burden in ALS

- Understanding QRL-201 and QRL-101 mechanisms for restoring STMN2 expression and reducing hyperexcitability-induced neurodegeneration in ALS, using FlexASO splicing technology to correct misplacing
- Unveiling preliminary trial results showcasing QRL-201 efficacy and safety data in humans
- Identifying potential subgroups that may benefit from more personalized approaches

Emma Bowden, Senior Vice President & Head of Development, **QurAlis**

12:00 **Lunch & Networking**

CONFERENCE DAY TWO - WEDNESDAY, MAY 14, 2025

ADVANCING BIOMARKER STRATEGIES: LINKING PRECLINICAL INSIGHTS, NEURO-METABOLIC DYSFUNCTIONS, & TRANSLATIONAL OPPORTUNITIES IN ALS & FTD

1:00 Development of a Novel, Orally Active, Brain Penetrable HDAC6 Inhibitors Shows a Therapeutic Potential for Amyotrophic Lateral Sclerosis (ALS)

- Determining if we can develop orally available peptides
- in vitro and in vivo evaluation of HDAC6 specific small molecule leads in ALS pre-clinical SOD1-G93A transgenic B6SJL.SOD1-G93A, in rNLS8 model of TDP-43 proteinopathy mouse models with PK/PD
- Evaluating biomarkers, safety pharmacology and familial SOD1, FUS, TDP-43 and C9orf72 genes from ALS patient samples

Hariprasad Vankayalapati, Chief Scientific Officer, **Biolexis Therapeutics**

1:30 Leveraging Biomarkers to Understand Mitochondrial Dysfunction in ALS: A Neuro-Metabolic Approach

- Exploring biomarkers reflecting mitochondrial energy imbalance in ALS with a focus on shifts from glucose to lipid oxidation
- Discussing early-stage biomarkers identified in ALS animal models (days 20-25), allowing for earlier detection of disease progression
- Showcasing preclinical data on metabolic interventions that restore glucose oxidation highlighting biomarkers used to monitor treatment effects
- Clinical data
- Expanding ALS biomarker panels beyond TDP-43 and NFL to improve early detection and disease monitoring
- Examining how metabolic biomarkers can inform clinical trial endpoints and improved disease progression tracking in ALS

John Nieland, Co-Founder & Chief Scientific Officer, **2N Pharma**

ADVANCING NEUROPROTECTION & IMMUNE MODULATION FOR IMPROVED OUTCOMES IN ALS & FTD

1:00 Session Reserved for Neuvivo

Vern Norviel, Chief Medical Officer, **Neuvivo**

1:30 Modulating the TREGs Pathway in ALS: Unveiling Innate Immunity's Role in Neurodegeneration

- To explore the role of regulatory T cell (Treg) dysfunction in neuroinflammation and as a contributing factor in the pathogenesis of ALS
- To discuss ex vivo and in vivo therapeutic approaches that target Treg dysfunction to suppress neuroinflammation and potentially slow disease progression in ALS and other neurodegenerative diseases
- To unveil plans for a phase 2 study evaluating COYA 302 in ALS

Jason Thonhoff, Head, Clinical Development, **Coya Therapeutics**

2:00 Afternoon Break & Refreshments

FUELLING MORE MEANINGFUL DRUG DEVELOPMENT TO MORE EFFECTIVELY MEET THE NEEDS OF INDIVIDUALS LIVING WITH ALS

2:30 **Preclinical & Early Clinical Development of NUZ-001: A Novel mTOR Inhibitor Demonstrating Potential as a Therapeutic Agent for Amyotrophic Lateral Sclerosis**

- ALS and related TDP-43 proteinopathies are driven by the mislocalization and aggregation of TDP-43, leading to neurodegeneration; the concurrent loss of Stathmin-2 (STMN2), a key regulator of axonal repair, further accelerates disease progression and highlights the need for therapies targeting both TDP-43 pathology and STMN2 restoration
- NUZ-001, a novel small-molecule mTOR inhibitor, is under investigation for its ability to reduce TDP-43 aggregation, restore STMN2 expression, and enhance autophagy—a critical protein clearance pathway—offering a multi-targeted approach to disease modification in ALS
- Preclinical studies using human iPSC-derived motor neurons showed that NUZ-001 significantly decreased cytoplasmic TDP-43 aggregation, increased STMN2 protein levels, and activated autophagy as indicated by elevated p62 accumulation and LC3 vesicle formation; functional assays further demonstrated neuroprotective effects including enhanced neurite outgrowth and preserved motor function
- Preliminary results from a Phase 1 clinical trial in ALS patients indicated that NUZ-001 is well tolerated with a favorable safety profile and pharmacokinetics, alongside early signs of efficacy and biomarker modulation, supporting further clinical development to explore its potential as a disease-modifying therapy

Michael Thurn
Chief Executive Officer
Neurizon

CONFERENCE DAY TWO - WEDNESDAY, MAY 14, 2025

3:00 **Engaging the Rare Patient Online**

- Practical approaches to reaching the rare disease community at large
- How we deliver unmatched value to our patient and industry partners
- A taste of the "secret sauce" behind our organic community growth;
- Case study, like for instance some of our learnings from the 2024 National Rare Disease Survey, with a spotlight on ALS or our new 2025 treatment survey

Ethan Ash
EVP, Business Development
Bionews

Marcella Debidda
President Patient Insights & Clinical Solutions
BioNews Clinical

3:30 Panel Discussion: **ALS Clinical Trials & Access to Investigational Therapies Through Expanded Access Programs: Perspectives from Patients, Caregivers, Advocacy & Biopharma, & How the Path to Diagnosis Shapes These Decisions**

- Exploring the path to receiving an ALS Diagnosis
- Highlighting the importance of early and meaningful patient involvement in shaping trial goals, eligibility criteria and logistics to ensure trials reflect patient priorities
- Unpacking the decision to participate in a clinical trial
- Balancing patient centricity and science in developing clinical trial protocols
- Discussing how to gather actionable insights from patients and PAGs to ensure input drives meaningful changes in trial processes
- Examining strategies to make trials more accessible to patients including geographical, financial and logistical barriers, while fostering open communication between researchers and participants
- Reshaping EAPs and OLEs with community-driven recommendations to ensure they meet the needs of patients and caregivers while advancing ALS research
- Highlighting how caregiver roles and patient input can shape policies to improve trial participation rates and strengthen trust within the ALS community

Deborah Bellina
ALS Advoacate & Caregiver to her son Matt

Mary Kay Turner
Senior Vice President, Global Patient Advocacy & Public Affairs
Brainstorm Cell Therapeutics

Ron Hoffman
Founder & Executive Director
Compassionate Care ALS

Wendy Hendrickson
Person Living with ALS
ALS Hope Foundation

4:20 **Chair's Closing Remarks**

4:30 **End of Conference**

"The program was well designed with the 2 critical paths/areas of interest. The speakers really had current and relevant experience in ALS development. In addition, the ALS patients and caregivers were a very nice touch, being able to interact with them, listen to their stories."

Executive Director, Clinical & Technical Operations, **Mitsubishi Tanabe Pharma**

2025 PARTNERSHIP OPPORTUNITIES

Network, Connect & Forge Lasting Connections & Collaborate with ALS & FTD Drug Developers

As *insitro* won $25M from **BMS** for reaching discovery milestones and selection of a first novel target for ALS, **Novo Nordisk** invested in **Eikonizo Therapeutics**, **Dewpoint Therapeutics** received $480M from **Mitsubishi Tanabe Pharma**, money is moving within ALS R&D, and drug developers are seeking strategic investments to advance their ALS pipelines

Do You Have What They're Looking For? Top Solutions Revealed:

In vitro models: 3D Cultures, hIPSCs & Brain Organoids to recapitulate the complexities of ALS pathology including TDP43 pathology, and disease heterogeneity, to ensure smoother translation of preclinical research to the clinic

In Vitro Models: including TDP43, and C9orf72 rodent models for translational research to better predict efficacy in humans

Biomarker Discovery: large-scale biomarker discovery platforms for early ID of novel biomarkers through comprehensive proteomic profiling

Genomic & Proteomic Solutions: including sequencing, genotyping and protein quantification

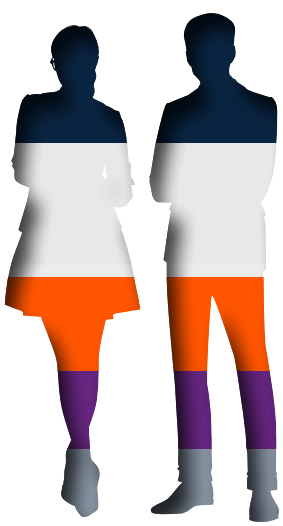
Diagnostic Tests: high sensitivity plasma and CSF assays for ALS for NFL and TDP43

Drug Discovery: high throughput AI & ML driven engines to support data-driven drug discovery for ALS

Clinical CROs: Diligent and efficient services as trusted and reliable partners sought by companies for trial design and program management. Establish how you build market trust and expand your customer base through streamlined and timely services

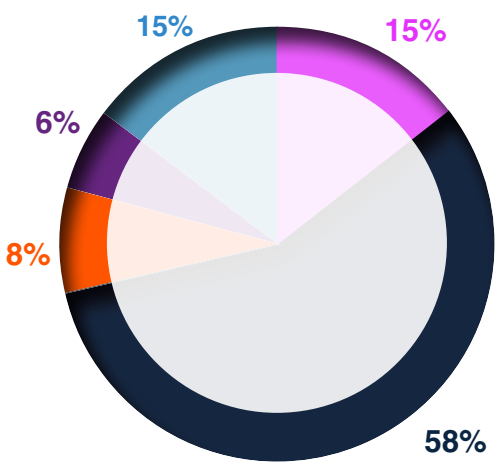
Partner with the **4th ALS Drug Development Summit** to elevate your brand and capitalize on our audience of senior decision-makers prioritizing ALS. With opportunities to deliver a presentation, network one-on-one with stakeholders to understand their needs first-hand, and enhance brand visibility. Contact us to explore a customized package that aligns with your business objectives and ensures your solution takes center stage.

SENIORITY OF ATTENDEES*



- C-Level/VP: 24%
- Director & Head: 33%
- Managers & Group Leads: 21%
- Scientist: 15%
- Other: 7%

TYPES OF COMPANIES ATTENDING*



*Statistics taken from the 3rd ALS Drug Development Summit

Contact

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Partnerships Director
Email: sponsor@hansonwade.com
Tel: +1 617 455 4188

2025 PARTNERS

QUANTERIX
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From discovery to diagnostics, Quanterix's ultrasensitive biomarker detection is fueling breakthroughs made possible through the Company's unparalleled sensitivity and flexibility. Its industry-leading Simoa® precision instruments, digital immunoassay technology, and CLIA-certified Accelerator laboratory have supported research that advances disease understanding, management, and patient care in neurology, oncology, immunology, cardiology, and infectious disease for nearly two decades.

www.quanterix.com

ANSWER ALS
EXPERTISE PARTNER

Answer ALS is the single largest & most comprehensive ALS research project in history, producing more ALS data than has ever been amassed, while openly sharing with the global research community. The program coordinates with over 20 institutions, establishing a critical foundation for new clinical trials and developing new ways to categorize ALS patients to identify specific druggable pathways in an effort to make ALS a treatable disease. Since opening the Answer ALS data repository, Neuromine, in 2021, over 300 independent ALS research projects have begun worldwide from free access to the rich data and available samples.

www.AnswerALS.org

ALAMAR BIOSCIENCES
EXPERTISE PARTNER

Alamar Biosciences is powering precision proteomics with automated, high throughput solutions for ultra-high sensitivity protein analysis across a range of multiplex levels in liquid biopsy samples. Our proprietary NULISA™ Chemistry utilizes a novel sequential capture and release method reducing background signal and increasing sensitivity and dynamic range compared with standard approaches. The NULISA Platform allows for a fully automated workflow with the ARGO™ System enabling less than 30 minutes hands-on time from sample to data.

www.alamarbio.com

NEURIZON
EXPERTISE PARTNER

Neurizon Therapeutics Limited, formerly known as PharmAust, is a clinical-stage biotechnology company dedicated to advancing treatments for neurodegenerative diseases. Neurizon is developing its lead drug candidate, NUZ-001, for the treatment of ALS, which is the most common form of motor neurone disease. Neurizon strategy is to accelerate access to effective ALS treatments for patients, while exploring NUZ-001's potential for broader neurodegenerative applications. Through international collaborations and rigorous clinical programs, Neurizon is dedicated to creating new horizons for patients and families impacted by complex neural disorders.

www.neurizon.com

ALS TDI
INDUSTRY PARTNER

The ALS Therapy Development Institute (ALS TDI) is world's largest non-profit research institute dedicated solely to ALS. To help accelerate global research and foster collaboration, we developed the ARC Data Commons. This platform allows researchers to easily explore de-identified data from our ALS Research Collaborative (ARC), the longest-running natural history study in ALS. This invaluable resource will enhance understanding of ALS patient subsets and fuel the discovery of potential treatments.

www.als.net

2025 PARTNERS

UNLEARN AI
PROGRAM PARTNER

Unlearn is a San Francisco-based technology company pioneering generative machine learning methods to eliminate trial and error in medicine. Unlearn's technology using patient digital twins is regulatory-qualified and used by leading global pharmaceutical companies to run AI-powered clinical trials that reach full enrollment faster and bring new treatments to patients sooner.

www.unlearn.ai

ELIXIRGEN SCIENTIFIC
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Elixirgen Scientific's purpose is to be at the forefront of medical innovation by harnessing the potential of iPSC differentiation technology. We exist to create limitless opportunities for advancing drug discovery and revolutionizing cell therapy across a multitude of diseases, including Alzheimer's Disease, Parkinson's Disease, rare neurodegenerative diseases, and many more. We are dedicated to delivering state-of-the-art, tailor-made solutions to empower the pharmaceutical industry towards the next great discovery.

www.elixirgensci.com

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HOSTING PARTNER

ARG is a contract research organization focused on oncology, immunology, and neurology, providing comprehensive clinical program development services ranging from planning to commercialization, achieving over 50 market authorizations to date. Founded in 2004 with the vision that every project should be highly individualized, ARG has experienced consistent growth across the globe, expanding our range to include drug and device strategic consulting, clinical trial management services, as well as clinical data and analytic solutions. ARG uses first-in-class technology platforms along with relationship-driven flexibility to optimize clinical studies because we believe everyone deserves to be well.

www.atlanticresearchgroup.com

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BrainXell provides high-purity, iPSC-derived human neurons and glia for research and development with a focus on drug discovery. Utilizing proprietary technology, we generate high-purity, subtype-specific neurons that mature rapidly and are quickly and easily ready for a variety of assays. Multiple neuron subtypes relevant to a range of disorders are available. Additionally, each neuron subtype can be made in custom batches from 50 million to 10 billion neurons from unique iPSC lines. We are dedicated to delivering the highest quality products for off-the-shelf neurons and custom service.

www.brainxell.com

TRANSPHARMATION
EXHIBITION PARTNER

Transpharmation is an accredited preclinical contract research organization specializing in translational pharmacology across various therapeutic areas. We offer neuroscience expertise, utilizing behavioral and molecular assays to help clients benchmark compounds. With extensive experience in antibiotic treatments and diet manipulations, we study their effects on the microbiome. Committed to on-time data delivery and providing flexible, customized solutions, we support clients in optimizing preclinical assets for the development of life-changing medicines.

www.transpharmation.com

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www.anatomic.com

AXOL BIOSCIENCE
EXHIBITION PARTNER

Axol Bioscience is the leading manufacturer of functional iPSC-derived cells and provider of outsourced services to support ALS drug discovery programs. We manufacturer functional iPSC-derived motor neurons, microglia and astrocytes, building co-culture model systems with relevant endpoints. iPSCs? What can we do to help.

www.axolbio.com

IXCELLS BIOTECHNOLOGIES
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iXCells Biotechnologies is a cell biology and cell technology company dedicated to providing innovative pre-clinical drug discovery solutions and disease relevant cellular models, enabling technologies and services to the academic, biotech and pharma communities to accelerate drug discovery. We offer high quality primary and iPSC-derived cells, custom iPSC services, functional bioassay development and execution. With a network of collaborators, we continue to make discoveries and expand our product offerings as a leader in the marketplace.

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www.catalent.com

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www.neurospector.com

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PROJECT MOSAIC
EVENT PARTNER

Project Mosaic is a collaborative R&D effort working to accelerate successful ALS drug development by equipping drug makers with a new class of patient-specific, sporadic ALS disease models ("Neurobiopsies™") designed to enable precision drug development.

www.projectmosaic.org

ALS NEWS TODAY
MEDIA PARTNER

ALS News Today shares credible, up-to-date science-backed news and information. They foster inclusive and supportive communities where patients and caregivers share experiences and access valuable resources. Over 50% of the Bionews team has a personal connection to rare diseases, fueling their dedication and understanding.

www.alsnewstoday.com

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DISCOVER how leading companies are pursuing novel targets, enhancing motor neuron cell models, modernizing clinical trial design, and navigating regulatory hurdles to develop more transformative treatments for sporadic and familial ALS

SHOWCASE your research to a diverse audience of 150+ ALS experts at the Scientific Poster Session on day one

ENGAGE in forward-thinking discussions around the potential of combination treatment regimens, peripheral immune targets, artificial intelligence and novel surrogate endpoints to transform ALS drug development

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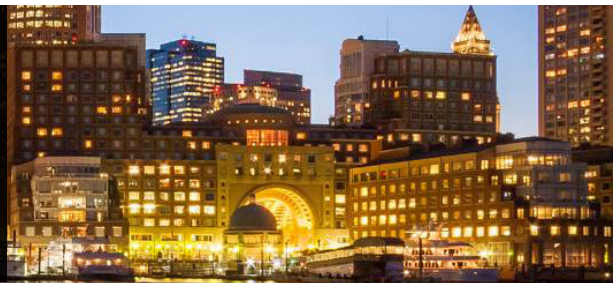

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