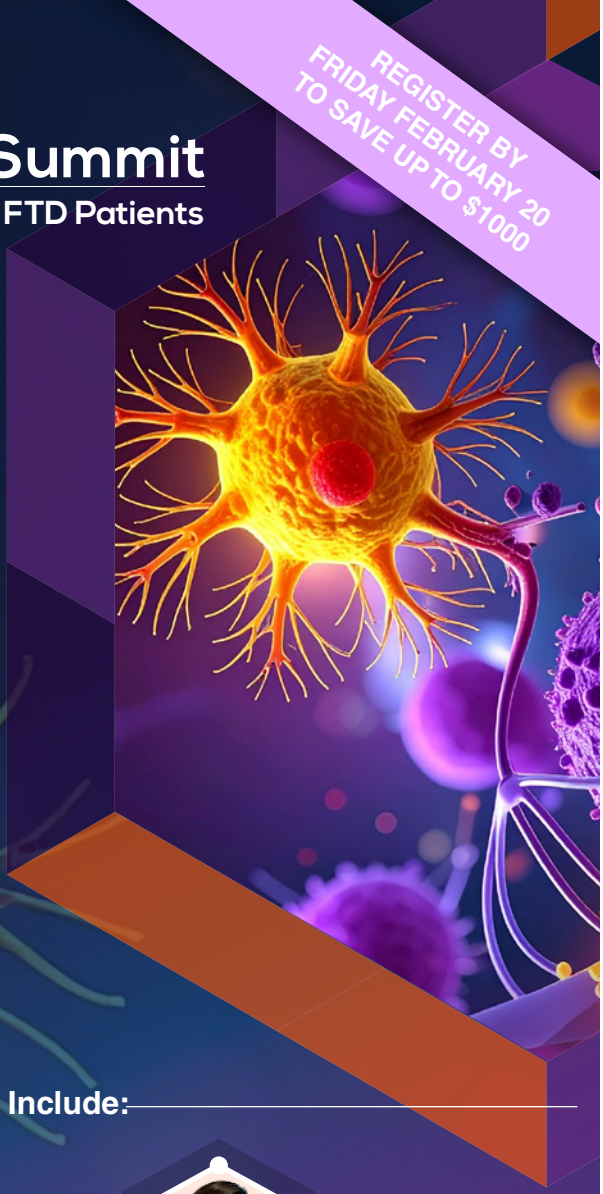
5th Annual

ALS Drug Development Summit

Accelerating Transformative Drugs to ALS & FTD Patients

REGISTER BY FRIDAY FEBRUARY 20 TO SAVE UP TO $1000

June 2-4, 2026 | Boston, MA



Bolstering a Resurgence in Novel Approaches to Target Underlying Pathology, Address Disease Heterogeneity & Restore Hope in a More Transformative Wave of ALS & FTD Therapies

Your 30+ Expert Speakers Include:

Arti Patel
Principal Scientist, Neuroscience & Program Lead
Novartis

Irina Antonijevic
Chief Medical Officer
Trace Neuroscience

Joyce Lo
Associate Director
Biogen

Kelly Glajch
Scientific Director, Rare Neurology-Neuroscience Therapeutic Area Unit
Takeda

Makoto Tamura
Executive Director of NeuroDiscovery Lab
Tanabe Pharma America

Michael Hayden
Chief Executive Officer
Prilenia

Philip C. Wong
Professor of Pathology
Johns Hopkins Medicine

Sandy Hinckley
Vice President & Head of Discovery
QurAlis

Yea Jin Kaeser-Woo
Senior Director
Eli Lilly

Proud To Partner With:

Evestia Clinical

BrainXell

ULYSSES NEUROSCIENCE

AXOL

als-drug-development.com
T +1 617 455 4188 **E** info@hansonwade.com

hansonwade

Reuniting a Community of ALS & FTD Stakeholders

As **Eli Lilly** further expands its ALS pipeline through **Alchemab** collaboration, **BMS** extends its partnership with insitro to discover new targets, and **Clene Nanomedicine** eyes regulatory submission, it's safe to say that the failures of the past two years have not dampened the appetite of biopharma. The field remains more dedicated than ever to addressing this increasingly urgent unmet need.

2025 has marked a renaissance of promising advances in modulating underlying genetics, protein aggregation, and neuroinflammatory mechanisms, truly moving the needle beyond traditional approaches to combat this devastating disease. The **5th ALS Drug Development Summit** returns this June to unite leading minds across biotech, pharma, academia and patient advocacy to showcase this progress, and pave the way to a cure.

Join 100+ leaders across discovery, translation, clinical, regulatory and external innovation from the likes of **AbbVie**, **AskBio**, **Biogen**, **Neurizon**, **Novartis**, **Prilenia Therapeutics**, **QurAlis**, **Takeda**, **Mitsubishi Tanabe Pharma**, **Trace Neuroscience**, **Sanofi** and **uniQure**, as well as PAGs, caregivers and academic KOLs, at the industry's only forum dedicated to translating scientific discoveries into the next wave of more targeted and efficacious therapies to ultimately restore hope for the ALS community.

Hear From Your 2026 Speakers:

> The summit will include, as in prior years, pertinent presentations related to current ALS drug development programs. It is a forum for open scientific, clinical and nonclinical exchanges of experiences in ALS drug development. Anyone developing drugs for ALS, can benefit from this focused conference and learn from others in the field. Given the rapidly changing landscape and regulatory perspectives, the drug development community will be strongest and most resilient when working together to develop meaningful new therapeutics for a devastating disease such as ALS

Irina Antonijevic, Chief Medical Officer, **Trace Neuroscience**

> Engaging with leading experts in a scientific field often sparks the kind of intellectual energy that gives rise to groundbreaking ideas

George Apostol, Head Clinical Development, **uniQure**

> This conference is a great opportunity for industry and academic experts to discuss the recent advances and remaining key challenges in the field

Kelly Glajch, Scientific Director, Rare Neurology- Neuroscience Therapeutic Area Unit, **Takeda**

Unmissable Highlights:

Unlocking Genetically Validated Targets to Drive ALS & FTD Therapeutics
Explore how cutting edge genomic insights are revealing high impact targets and modifier genes across ALS and FTD, with leaders from **Novartis**, **QurAlis**, and **Biogen** decoding shared disease mechanisms and guiding next generation drug discovery.

Modelling Disease Heterogeneity with Advanced *In Vitro* Platforms
Dive into the latest innovations in iPSC derived motor neurons, 3D neuronal cultures, and high content screening tools that are transforming ALS research, with **Takeda** and leading academic labs showcasing translational breakthroughs.

Learning from Past Failures to Refine Clinical Innovation
Unpack hard won lessons from recent trials to improve endpoint selection, trial sensitivity, and regulatory engagement. Hear from **Johns Hopkins**, and **NIH** on how to design more meaningful and inclusive studies for people living with ALS.

Embedding the Patient Voice to Shape Trial Design & Access
Discover how patient reported outcomes, digital biomarkers, and advocacy partnerships are redefining what matters most in ALS drug development, with insights from **Everything ALS**, **CPATH**, and **Tufts Medical Center**.

Accelerating ALS Investment & Strategic Collaborations
Connect directly with investors and pharma leaders to understand what drives funding decisions in ALS and FTD. **AbbVie**, **Trace Neuroscience**, and **RA Capital** reveal how early data, biomarkers, and patient centered endpoints are reshaping the investment landscape.

Harnessing Spatial Omics & AI to Revolutionize Target Discovery
See how spatial transcriptomics, proteomics, and AI powered platforms are uncovering novel ALS pathways and biomarkers, with **Eli Lilly**, **Verge Genomics**, and **AC Immune** leading the charge in precision medicine.

100+ Global Attendees from Across the Renal Landscape

10+ Hours of Face-to-Face Networking Opportunities

2 Dedicated Tracks of Content for the Whole Team

30+ Expert Speakers with Unique Insights

3 Days of Unmissable Data-Driven Content

als-drug-development.com
T +1 617 455 4188 **E** info@hansonwade.com

hansonwade

2026 Speaker Faculty

5th Annual
ALS Drug Development Summit
Accelerating Transformative Drugs to ALS & FTD Patients
June 2-4, 2026 | Boston, MA

Pharma Powerhouses Driving Neuromuscular Innovation

Arti Patel
Principal Scientist, Neuroscience & Program Lead
Novartis

Francisca Martinez-Raub
Director
Eli Lilly

Joel Schwartz
Director, Scientific Digital Biomarkers Neuroscience
Bristol Myers Squibb

Joyce Lo
Associate Director
Biogen

Kelly Glajch
Scientific Director, Rare Neurology-Neuroscience Therapeutic Area Unit
Takeda

Ivan Nyarko-Danquah
Postdoctoral Fellow
Sanofi

Makoto Tamura
Executive Director of NeuroDiscovery Lab
Tanabe Pharma America

Murali Gopalakrishnan
Global Head, Neuroscience Search & Evaluation, Corporate Strategy Officer
AbbVie

Yea Jin Kaeser-Woo
Senior Director
Eli Lilly

Yunfeng Huang
Principal Scientist
Biogen

Biotech Trailblazers Transforming ALS & FTD Therapeutics

Christopher Shaw
Chief Scientific Officer & Clinical Advisor
AviadoBio

George Apostol
Head of Clinical Development
UniQure

Irina Antonijevic
Chief Medical Officer
Trace Neuroscience

Javier San Martin
Chief Medical Officer
LeonaBio

Mansuo Lu Shannon
Chief Scientific Officer
Ask Bio

Michael Hayden
Chief Executive Officer
Prilenia

Michael Thurn
Chief Executive Officer
Neurizon

Sandy Hinkley
Vice President, Head of Discovery
QurAlis

Sarah Knutson
Executive Director, Early Discovery
ROME Therapeutics

Investors, Advocates & Strategic Voices

Christian Rubio
Executive Director
Everything ALS

Eloise Jarvis
Senior Researcher
Beacon Research Operations

Hanxiong (Bear) Zhang
Principal
RA Capital Management

2026 Speaker Faculty

5th Annual
ALS Drug Development Summit
Accelerating Transformative Drugs to ALS & FTD Patients
June 2-4, 2026 | Boston, MA

Academic Titans & Clinical Thought Leaders

Colin Hovingay
Vice President of Rare & Orphan Diseases
Critical Path Institute

John Replogle
Director
National Institutes of Health (NIH)

Philip C. Wong
Professor of Pathology
John Hopkins Medicine

Ruth Chia
Staff Scientist
National Institutes of Health (NIH)

CRO Innovators Accelerating CNS Research

Ignazio Di Giovanna
Vice President, Scientific Affairs, Neurology
Evestia Clinical

Massimiliano Bianchi
Founder, President & Chief Executive Officer
Ulysses Neuroscience

Matthew Mandeville
Senior Business Development Manager
BrainXell

"Such a great conference. I loved that the speakers were well balanced between early rising stars and more experienced researchers"

Co-Founder, Chief Scientific Officer & Head of Biology, **2N Pharma**

"There was a great range of content. The last panel discussion with a person living with ALS and with advocates was great"

Associate Director, **Alnylam Pharmaceuticals**

"I really enjoyed the balance between academic and industry presentation. The atmosphere was very lively and the size of the meeting just right to be able to interact with everyone while having representatives from a wide variety of fields involved in ALS"

Director, **AviadoBio**

Pre-Conference Workshop Day
Tuesday June 2, 2026

5th Annual
ALS Drug Development Summit
Accelerating Transformative Drugs to ALS & FTD Patients
June 2-4, 2026 | Boston, MA

Morning Check-In: *Served with coffee & light breakfast* 8.00

Workshop **A** 9.00

Deciphering the TDP-43 Driven Genetic Overlap of ALS & FTD Towards NextGeneration Therapies

This workshop will explore the shared genetic and molecular mechanisms underlying Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia (FTD), providing insights into converging pathways that may inform novel therapeutic strategies. Participants will examine the latest discoveries in disease-causing mutations, modifier genes, and cryptic exon biology, and discuss how these findings are guiding target identification and translational research. The session will also cover approaches to leverage genetic insights for biomarker development, patient stratification, and the design of mechanism-based clinical trials. Attendees will gain a clearer understanding of the genetic intersections between ALS and FTD and the implications for next-generation therapies.

Key Discussion Points

- Genetic overlap between ALS and FTD and how shared pathways contribute to disease progression
- Insights from discovery programs and translational research into novel targets and modifier genes
- Integration of genetic findings into biomarker development and patient stratification for clinical trials
- Opportunities and challenges in leveraging genetic insights to design mechanism-based therapies across ALS and FTD

Workshop Leaders:

Arti Patel
Principal Scientist,
Neuroscience & Program Lead
Novartis

Francisca Martinez-Raub
Postdoctoral Scientist
Eli Lilly

Morning Refreshment & Networking 11.00

Workshop **B** 11.15

Developing More Meaningful Endpoints through Digital Measures & Patient Reported Outcomes (PROs) to Capture What Matters Most to ALS Patients in Clinical Trials & Drug Design

This interactive workshop will explore how clinical trial endpoints can be designed to reflect what truly matters to people living with ALS. Participants will discuss strategies for integrating patient-reported outcomes, functional measures, digital biomarkers, and quality-of-life metrics into trial design. The session will highlight collaborative approaches with patient advocacy groups, lessons from ongoing studies, and regulatory considerations for demonstrating clinical meaningfulness. Attendees will gain practical insights into creating endpoints that enhance trial relevance, patient engagement, and therapeutic impact.

Key Discussion Points

- Defining endpoints that capture functional, cognitive, and quality-of-life outcomes meaningful to patients
- Incorporating patient-reported outcomes, digital tools, and real-world data into trial design
- Regulatory perspectives on patient-centered endpoints and demonstrating clinical relevance
- Collaborative models to co-design endpoints with patients and advocacy groups

Workshop Leaders:

Christian Rubio
Executive Director
Everything ALS

Colin Hovingay
Vice President of Rare & Orphan Diseases
CPATH

Joel Schwartz
Scientific Director
Bristol Myers Squibb

Lunch & Networking 1.15

Pre-Conference Workshop Day
Tuesday June 2, 2026

Workshop C — 2:15

Next-Generation *In Vitro* Platforms for ALS Drug Development & Target Validation

This workshop will explore the latest *in vitro* tools and model systems used to advance ALS drug discovery and target validation. Participants will discuss how human-derived cell models, induced pluripotent stem cells (iPSCs), and advanced 3D neuronal cultures are being leveraged to study disease mechanisms, screen therapeutic candidates, and identify biomarkers. The session will highlight best practices for experimental design, reproducibility, and translational relevance, as well as emerging technologies that enhance the predictive power of *in vitro* studies. Attendees will gain practical insights into selecting and applying these tools to accelerate ALS research and development.

Key Discussion Points

- Overview of current *in vitro* models used in ALS research, including iPSC-derived motor neurons and glial co-cultures
- Strategies for using *in vitro* systems to validate targets, test small molecules, genetic therapies, and combination approaches
- Approaches to improve reproducibility, scalability, and translational relevance in ALS *in vitro* studies
- Emerging technologies and high-content screening platforms to accelerate discovery and preclinical testing

Workshop Leaders:

Chris Shaw
Chief Scientific & Clinical Advisor/Co-Founder
AviadoBio

Ivan Nyarko-Danquah
Postdoctoral Fellow
Sanofi

Kelly Glajch
Scientific Director, Rare Neurology-Neuroscience Therapeutic Area Unit
Takeda

End of Pre-Conference Workshop Day — 4:15

"Great interactivity; all cutting-edge 'hot topics' were in."
Global Program Head Neurology, **Duke University**

"Well structured schedule, balanced between clinical and preclinical with lots of opportunities to discuss and network."
Senior Director – CNS Program Research, **Envisagenics**

"Excellent speakers. Lots of networking time and a very friendly crowd."
Owner, **JT Pharma**

"Collaborative, informative, and perfect length of time."
VP Operations, **Repeat Therapeutics**

Conference Day One
Wednesday June 3, 2026

7:30 **Morning Check-In:** *Served with coffee & light breakfast*

8.25 **Chair's Opening Remarks**

Targeting ALS at Its Earliest & Most Actionable Biology: From Upstream Disease Mechanisms & Genetic Precision to Biomarker-Guided Prevention & Regulatory Pathways

8.30 Panel Discussion: **How the Industry is Leveraging Spatial Omics for ALS Target Discovery & Biomarkers?**

- How is spatial omics transforming our understanding of ALS pathology compared with traditional bulk tissue or single-cell approaches?
- Which emerging ALS targets or molecular pathways have been most compellingly revealed through spatial transcriptomics, proteomics, or multi-omic integration?
- How can spatial omics data be translated into actionable biomarkers for clinical trials, including patient stratification, early detection, or monitoring therapeutic response?
- What are the key technical, analytical, and interpretational challenges in applying spatial omics to human ALS tissue, and how are industry and academia addressing these?
- How do collaborative approaches between pharma, academic centers, and genome institutes accelerate the path from spatial omics discovery to drug development?
- Looking forward, what role will AI and machine learning play in extracting meaningful insights from complex spatial omics datasets in ALS?

Joyce Lo
Associate Director
Biogen

Yea Jin Kaeser-Woo
Senior Director
Eli Lilly

9.00 **Restoring UNC13A Function in ALS: Translating *In Vivo* ASO Biology into Clinical Impact**

- First *in vivo* evidence that ASO-mediated restoration of UNC13A cryptic exon function can modify disease-relevant biology, validating UNC13A as a therapeutically actionable target in ALS
- Why partial restoration of UNC13A may be sufficient to move the needle clinically, and how to think about biological sufficiency versus complete target correction
- The critical importance of early intervention, with data supporting delivery before irreversible neuronal loss to maximise therapeutic benefit
- The need for a companion biomarker strategy to guide patient selection and timing in clinical trials, ensuring the right patients receive treatment at the right stage

Philip C. Wong
Professor of Pathology
Johns Hopkins Medicine

9.30 Panel Discussion: **What Happens Upstream of TDP-43 Misfolding? Shifting Focus from Restoring Function to Understanding Root Cause of Aggregate Pathology**

- What are the earliest cellular events that trigger TDP-43 mislocalization and aggregation, and how do these upstream mechanisms vary across ALS subtypes?
- How do general protein homeostasis pathways, including nuclear import/export and stress response mechanisms, influence TDP-43 pathology?
- Which cryptic exons, epigenetic changes, or C9orf72-related mechanisms are likely contributors to disease initiation, and how should these inform target prioritization?
- How can we improve ALS model systems to better reflect the physiological context of TDP-43 misfolding and aggregate formation?
- Moving beyond restoring TDP-43 function, what strategies can drug developers adopt to intervene at the root cause of aggregate pathology?

Philip C. Wong
Professor of Pathology
Johns Hopkins Medicine

Ruth Chia
Staff Scientist
National Institutes of Health (NIH)

10.00 **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 space. Designed to maximize your introduction to numerous new individuals and serve as a catalyst for ongoing discussions during the Summit.

10.30 **Morning Break & Refreshments**

Conference Day One
Wednesday June 3, 2026

5th Annual
ALS Drug Development Summit
Accelerating Transformative Drugs to ALS & FTD Patients
June 2-4, 2026 | Boston, MA

TRACK A: DISCOVERY, PRECLINICAL & TRANSLATIONAL

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

Deciphering ALS Genetic Underpinnings to Illuminate Fast & Slow Progressors in ALS

11:00 Leveraging Modifier Genes & Molecular Signatures to Stratify Fast Vs Slow Progressors in ALS

- Characterizing variants such as those in UNC13A that shape both ALS susceptibility and disease trajectory, influencing rate of decline and survival
- Identifying emerging risk and modifier genes through integrated GWAS, WGS, splicing-QTL, and rare-variant analyses to illuminate new mechanisms driving heterogeneity
- Improving progression modeling by addressing limitations of ALSFRS-R, incorporating survival data, and integrating biomarkers like longitudinal neurofilament levels to better link genotype with phenotype
- Prioritizing genetically validated progression modifiers as compelling targets for disease-modifying therapies with broad patient impact

Yunfeng Huang, Principal Scientist, **Biogen**

11:30 A Plasma Proteomic Biomarker Panel for Early Detection & Disease Prediction in ALS

- Utilizing high throughput plasma proteomics to define a 33 protein biomarker panel that differentiates ALS from controls and other neurological diseases with high classification accuracy
- Applying machine learning to integrate plasma protein levels with clinical features, achieving robust diagnostic performance (AUC ~98%) and a composite ALS risk score
- Demonstrating that shifts in the proteomic risk score emerge years (up to ~10 yrs) before clinical symptom onset, highlighting a prodromal phase amenable to early detection and intervention
- Discussing how multi protein signatures and pathway insights (skeletal muscle, neuronal and energy metabolism processes) can inform prognostic stratification and future therapeutic targeting, including broader validation and global applicability

Ruth Chia, Staff Scientist, National Institutes of Health (NIH)

TRACK B: CLINICAL & REGULATORY

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

Leveraging Imaging & Digital Biomarkers for Improved Disease Monitoring & Assessment of Patient Benefit

11:00 Sigma-1 Receptor Agonism in ALS: Why, How & When?

- Explaining why sigma-1 receptor agonism is an important target for ALS, including human genetic data
- Providing *in vitro*, *in vivo* and human data to support this
- How this data and prior Phase 2 data encouraged design of Phase 3 pivotal trial, including novel endpoints such as speech
- Timelines, milestones and potential outcomes, and providing insights from ongoing NIH-funded expanded access program (EAP)
- Similar mechanisms may highlight sigma-1 agonism for other neurodegenerative disorders, such as FTD

Michael Hayden, Chief Executive Officer, **Prilenia**

11:30 The Roadmap to Operationalizing AI & Digital Biomarkers in ALS Care

- Designing compliant, trustworthy AI in ALS: how companies balance regulatory rigor with rapid innovation in real world clinical and research settings
- Enabling hyper personalization through AI and digital biomarkers, using continuous, multimodal data to reflect individual disease trajectories rather than population averages
- Creating AI powered educational feedback loops that translate patient generated data into meaningful insights for patients, caregivers, and clinicians
- Leveraging tech enabled non profits as a deployment model, aligning patient trust, open collaboration, and mission driven AI innovation to accelerate impact in ALS

Christian Rubio, Executive Director, **Everything ALS**

12.00 **Lunch & Networking**

Conference Day One
Wednesday June 3, 2026

5th Annual
ALS Drug Development Summit
Accelerating Transformative Drugs to ALS & FTD Patients

June 2-4, 2026 | Boston, MA

From Nucleic Acid Therapies & Spatial Omics to Advanced Models for Precision Drug Discovery

1:00 Advancing Nucleic Acid Therapies for ALS: Delivery Innovations, Multiomic Insights, & Next-Generation Target Discovery

- Learnings from full-cycle oligonucleotide development in ALS, spanning target validation, lead identification, and clinical candidate nomination
- Delivery innovations and nucleic-acid–based strategies that may improve therapeutic reach and durability in neurodegenerative disease
- How integrated data science, including bioinformatics, computational biology, and biomarker discovery, is shaping next-generation ALS programs
- The role of multiomic and spatial biology platforms in enabling precision medicine approaches for ALS and other neurodegenerative disorders

Yea Jin Kaeser-Woo, Senior Director, **Eli Lilly**

1:30 Advancing ALS Drug Discovery with iPSC-Derived Neurons & Glia

- Senior Business Development 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 harbouring 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**

1:40 Cracking ALS & FTD: Translating Human Disease Biology into a Discovery Pipeline

- Why ALS and FTD demand a discovery first rethink, and how deep integration of genetics, clinical phenotypes, biomarkers, and longitudinal data reveals disease driving biology missed by traditional approaches
- Using human data to drive target identification and prioritisation, grounding ALS & FTD pipelines in patient relevant mechanisms from the outset
- Bridging ALS–FTD biology to inform shared and divergent pathways, and how this insight shapes program strategy, indication selection, and translational confidence
- Building a scalable discovery engine for neurodegeneration, where integrated platforms accelerate progression from biological insight to pipeline ready therapeutic hypotheses

Sandy Hinckley, Vice President, Head of Discovery, **QurAlis**

2:10 TDP-43 LOF–Driven Splicing Signature as a Molecular Classifier for ALS

- Demonstrate how a TDP-43 loss-of-function–driven alternative splicing signature functions as a mechanism-based molecular classifier that accurately distinguishes ALS from healthy controls
- Outline the machine-learning framework built on iPSC and patient spinal-cord transcriptomes, emphasizing feature interpretability and reproducible performance across independent ALS cohorts
- Highlight the implications of these findings for developing mechanism-anchored biomarkers and identifying novel therapeutic targets in ALS

Makoto Tamura, Executive Director of NeuroDiscovery Lab, **Tanabe Pharma America**

Redefining Biomarkers & Trial Design to Accelerate Neurodegenerative Disease Therapies

1:00 Targeting Microglial TREM2 to Shift ALS Neuroinflammation: Insights from the ASTRALS VHB937 Phase 2 Study

- The biological rationale for modulating microglial TREM2 with VHB937 to potentially enhance protective microglial functions and support motor neuron health in ALS
- Key design features of the ASTRALS Phase 2 trial, including the 40 week double blind period followed by an open label extension to assess long term safety and functional outcomes
- Early clinical observations from the ongoing study and how proximal signals will inform future ALS therapeutic strategies
- The role of functional endpoints like ventilator free survival and ALSFRS R changes in interpreting disease modifying potential in a TREM2 targeted ALS therapy

Arti Patel, Principal Scientist II, Program Lead & Lab Head in Neuroscience, Biomedical Research, **Novartis**

1:30 Session Reserved for Ulysses Neuroscience

ULYSSES NEUROSCIENCE

Massimiliano Bianchi, Founder, President & Chief Executive Officer, **Ulysses Neuroscience**

1:40 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

Ignazio Di Giovanna, Vice President, Scientific Affairs, Neurology, **Evestia Clinical**

2:10 Advancing ATH-1105 in ALS: Strategic Trial Design, Biomarker Integration & Adaptive Methodologies

- How refinements to the ALS program, including ATH-1105's mechanism as a CNS-penetrant positive modulator of the neurotrophic HGF system, are shaping a larger, more globally inclusive Phase 2-ready design
- Integrating established biomarkers such as neurofilament light (NfL) and exploratory endpoints into trial architecture to align with the biological effects anticipated from ATH-1105
- Leveraging AI-supported models and external control arms to enhance signal detection and improve statistical power in a non-gene therapy small molecule ALS study
- Designing trials that reflect the heterogeneity of sporadic and familial ALS while aligning clinical outcome assessments with ATH-1105's neuroprotective profile and translational rationale

Javier San Martin, Chief Medical Officer, **LeonaBio**

Conference Day One
Wednesday June 3, 2026

2:40 **Afternoon Break & Refreshments**

Overviewing the Latest Trends, Advancements & Predictions for the Future of ALS R&D

3:40 **A Landscape Review of ALS Therapy Development**

- The drug landscape, displaying critical data-driven therapeutic and clinical trial highlights, as well as current trends
- A look at the developer landscape, allowing for understanding of the commercial market involving these drugs' development
- The outlook for the future of the ALS therapeutics field from a preclinical and clinical perspective

Eloise Jarvis
Senior Researcher
Beacon Research Operations

Fueling More Meaningful Drug Development to More Effectively Meet the Needs of Individuals Living with ALS

4:10 **Panel Discussion: Putting Patients First in ALS Research Advancing Clinical Insights & Care Through Engagement**

- How patient-reported outcomes, quality-of-life metrics, and digital tools are shaping trial design, endpoint selection, and real-world evidence in ALS studies
- The role of patient advocacy groups and community engagement in prioritizing unmet needs, accelerating recruitment, and informing study feasibility
- Lessons from participatory research programs highlighting patient perspectives on symptom tracking, trial burden, and therapy acceptability
- Strategies for integrating patient insights into regulatory submissions, biomarker development, and therapeutic prioritization, ensuring research translates into meaningful impact for people living with ALS

Christian Rubio
Executive Director
Everything ALS

4:40 **Chair's Closing Remarks**

4:45 **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.

5:45 **End of Conference Day One**

"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**

"I really enjoyed the interactive sessions this year, and the many opportunities to interact with people working in and living with ALS by design. This really enriched the experience and I thought it was an appropriate size conference to have those kinds of sessions"

Senior Director of Biomarker Development, **Neumora**

Conference Day Two
Thursday June 4, 2026

8.30 **Morning Check-In:** *Served with coffee & light breakfast*

9.25 **Chair's Opening Remarks**

From Capital to Clinic: Enabling ALS & FTD Progress Through Investment & TDP-43 Insights

9.30 **Panel Discussion: Revitalizing the ALS Investment Landscape: Opportunities, Challenges, & the Path Forward**

- What are the current barriers to investment in ALS therapeutics, and how can stakeholders address perceived scientific or clinical risks?
- Which emerging modalities or targets in ALS are attracting the most investor interest, and where do pharma companies see the greatest potential for collaboration?
- How can early-stage data, biomarkers, or patient-focused endpoints help de-risk ALS programs for investors and facilitate pharma partnerships?
- What lessons can be learned from past ALS investment successes and failures to guide more sustainable funding strategies?
- How can collaboration between VCs, corporate pharma, and patient advocacy groups accelerate both funding and development of ALS therapies?

Hanxiong (Bear) Zhang
Principal
RA Capital Management

Murali Gopalakrishnan
Global Head, Neuroscience Search & Evaluation, Corporate Strategy Office
AbbVie

10.00 **Panel Discussion: How Can We Treat Underlying TDP43 Pathology Outside of Clinical Presentation to Develop Effective Treatments in ALS, FTD & Beyond?**

- What is the current understanding of TDP-43 pathology across ALS, FTD, and related neurodegenerative diseases, and how does it inform therapeutic strategies?
- How can therapies be designed to target upstream TDP-43 mislocalization, aggregation, or loss-of-function, independent of clinical symptom presentation?
- The role of biomarkers and imaging in detecting subclinical TDP-43 pathology and enabling earlier intervention in patients
- Challenges and opportunities in translating preclinical findings into human studies, including target engagement, dosing strategies, and safety considerations
- Potential combinatorial approaches, such as pairing TDP-43-targeted therapies with neuroprotective or gene-modulating modalities to enhance efficacy

Irina Antonijevic
Chief Medical Officer
Trace Neuroscience

Kelly Glajch
Scientific Director, Rare Neurology- Neuroscience Therapeutic Area Unit
Takeda

10.30 **Morning Break & Refreshments**

Innovating Beyond TDP-43 Unlocking Novel Mechanisms to Combat ALS & Neurodegeneration

11:00 Targeting LINE-1 Retrotransposon Activity to Address Neurodegeneration & Inflammation in ALS

- LINE-1 retrotransposons can become aberrantly active under stress, contributing to genomic instability and neuroinflammation in ALS
- Selective LINE-1 reverse transcriptase inhibitors suppress cytosolic LINE-1 activity and blunt innate immune activation
- Preclinical models show neuroprotective effects, supporting motor neuron survival and reducing pathological signaling
- Translational considerations include biomarker development, CNS delivery, and differentiating selective inhibitors from earlier non-specific approaches

Sarah Knutson, Executive Director, Early Discovery, **ROME Therapeutics**

Restoring TDP-43 Function and Reducing Disease Burden Through Cutting-Edge Genetic Therapies

11:00 From Target Validation Through Nonclinical Development to First-in-Human: Lessons Learned Developing an ASO Therapy for Most People Living with ALS

- Key learnings from nonclinical development of an ASO intended for nearly all people with ALS, including validation of potency, durability, and CNS biodistribution
- How mitigation of dose-dependent ASO side effects informed dosing strategy, injection frequency, and overall clinical trial design
- Presenting first-in-human clinical data, including early safety, PK, and target-engagement insights
- Insights from regulatory interactions shaped by the unique profile of the ASO and the requirements for first-in-human advancement

Irina Antonijevic, Chief Medical Officer, **Trace Neuroscience**

Conference Day Two
Thursday June 4, 2026

11:30 The Intersection of TMEM106B Fibrils, TDP-43 Pathology & Lysosomal Function in Neurodegeneration

- How TMEM106B genetic variants modulate ALS–FTD risk and intersect with progranulin-dependent lysosomal biology
- Developed a model system of TMEM106B aggregation that leads to intra-lysosomal fibril formation in human neurons
- Evidence that TMEM106B aggregation induces elevated levels of phospho-TDP-43
- Direct co-localization of TMEM106B fibrils and TDP-43 aggregates within individual lysosomes
- Implications of TMEM106B-driven lysosomal failure as an upstream, targetable mechanism across neurodegenerative diseases

John Replogle, Postdoctoral Fellow, **National Institutes of Health (NIH)**

11:30 Advancing AMT-162 Gene Therapy for SOD1-ALS Clinical Progress & Future Opportunities

- Overview of AMT-162, a one-time intrathecal gene therapy designed to silence SOD1 expression in patients with SOD1-linked ALS and the rationale for targeting this mutation
- Early clinical progress from the Phase I/II EPISOD1 trial, including first patient dosing and favorable independent safety data supporting progression to the second dose cohort
- Key exploratory efficacy and biomarker strategies in the AMT-162 program, including use of neurofilament light and SOD1 protein levels to assess potential impact on disease progression
- Challenges and opportunities in gene therapy for ALS, including regulatory considerations, patient selection, and the potential for one-time treatment paradigms in rare genetic subtypes

George Apostol, Head of Clinical Development, **UniQure**

12.00 **Lunch & Networking**

Pioneering Precision ALS Therapies from Vectorized Antibodies to Personalized & Dual-Modality RNA Strategies

1:00 Revisiting Gene Silencing in ALS: From SOD1 Biology to Next-Generation CNS RNA Platforms

- Re-examining SOD1 as a therapeutic target in ALS using updated gene-silencing and delivery approaches informed by recent preclinical work
- Development of a physiological miRNA-based gene silencing platform using multi-guide designs to modulate single genes or ALS-relevant pathways
- Preclinical efficacy and expression data in ALS mouse models, including opportunities for combining gene silencing with protein supplementation
- Engineering a BBB-crossing capsid to enable durable, pan-CNS delivery of RNA therapeutics for ALS and related neurodegenerative diseases

Chris Shaw, Chief Scientific & Clinical Advisor/Co-Founder, **AviadoBio**

Transforming Clinical ALS Treatment with Novel Modalities Targeting Proteinopathy, Neuroinflammation & Neuronal Resilience

1:00 Advancing NUZ-001 in ALS: Targeting TDP-43 Biology Through Adaptive Clinical Development

- The evolving understanding of TDP-43 pathology in ALS and how insights into protein aggregation and neuronal stress informed the development rationale for NUZ-001
- Preclinical and early translational findings indicating that NUZ-001 functions as a stress-adaptive modulator, supporting cellular protein quality-control processes, including proteasomal function and autophagy, to reduce neuronal stress associated with aggregated proteins such as TDP-43
- Clinical development of NUZ-001 within the HEALEY ALS Platform Trial, with discussion of the trial structure, rationale for the adaptive platform design, and how such approaches support efficient evaluation of investigational therapies in ALS
- Interpreting preliminary clinical results to date and the role of the open-label extension in strengthening the evidence base for therapeutic effectiveness
- Upcoming development milestones and data readouts, and how trial design considerations, biomarker strategy, and data maturity shape confidence in signal detection for disease-modifying ALS therapies

Michael Thurn, Chief Executive Officer, **Neurizon**

> "The breadth of the meeting, from preclinical to clinical research, is very enjoyable. The perspectives from patients and patient advocates are also valuable experience."
>
> Assistant Professor, **Harvard Medical School**

Conference Day Two
Thursday June 4, 2026

1:30 A 30,000 Foot View of TDP43 Direct Targeting Strategies – A Thought Experiment

- Overview of TDP-43 pathology as the central disease hallmark in the majority of ALS patients
- Current therapeutic approaches targeting TDP-43 loss of nuclear function
- Strategies aimed at mitigating toxic gain of function, aggregation, and cytoplasmic mislocalization
- Key lessons from the field, remaining translational challenges, and future directions for TDP-43–targeted therapies

Joyce Lo, Associate Director, **Biogen**

1:30 Roundtable Discussion: Advancing Combination Therapies in ALS: Integrating Small Molecules & Genetic Medicines for Greater Therapeutic Impact

- Scientific rationale for combining small-molecule neuroprotective agents with genetic medicines to target multiple ALS disease pathways, including RNA dysregulation and motor neuron degeneration
- Lessons from preclinical and early clinical PrimeC data showing enhanced efficacy and biomarker modulation with multi-component combination strategies versus monotherapies
- Regulatory considerations for combination therapies, including trial design, biomarker endpoints, and dual-mechanism product development
- Patient and advocacy perspectives on the benefit–risk profile of oral multi-target combinations, and opportunities for industry collaboration to accelerate adoption

2.00 **Afternoon Break & Refreshments**

Building Tomorrow's ALS Drug Development Framework: Synergistic Approaches & Accelerated Pathways

2.30 **Panel Discussion: Blending Modalities for Complex Neurodegeneration: What Will It Take to Treat ALS?**

- How regulatory expectations differ across modalities (oral small molecules, ASOs, viral gene therapies, RNA medicines, cell therapies) and how developers can prepare for emerging FDA/EMA frameworks in ALS
- Which modalities are best suited for distinct ALS biological subtypes, including TDP-43 proteinopathy, C9orf72 repeat expansion, inflammatory/neuroimmune signatures, and mitochondrial/metabolic dysfunction
- Opportunities and challenges for combining modalities—for example coupling gene modulation with neuroprotective small molecules, pairing oligos with small-molecule chaperones, or integrating metabolic stabilizers with anti-inflammatory approaches
- How modality mechanisms intersect (protein homeostasis, RNA regulation, axonal transport, synaptic resilience) and how cross-mechanistic synergy could inform trial design or biomarker development
- What is needed to run smarter, modality-agnostic clinical trials—harmonized biomarkers (NfL, speech, digital mobility), shared control arms, adaptive designs, and platform/basket approaches

Mansuo Lu Shannon
Chief Scientific Officer
Ask Bio

Sarah Knutson
Executive Director, Early Discovery
ROME Therapeutics

3.00 **Chair's Closing Remarks**

3:05 **End of Conference Day Two**

"Very relevant topics. Awesome and knowledgeable presenters. One of the best ALS meetings I have been to"

Vice President Clinical Development, **PTC Therapeutics**

2026 Partners

Evestia Clinical
Hosting Partner

Evestia Clinical 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, Evestia Clinical 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. Evestia Clinical uses first-in-class technology platforms along with relationship-driven flexibility to optimize clinical studies because we believe everyone deserves to be well.

www.evestiaclinical.com

BrainXell
Innovation Partner

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

Ulysses Neuroscience
Innovation Partner

At Ulysses Neuroscience, we stand at the forefront of innovation as your vanguard CRO and translational partner, dedicated to advancing the understanding and treatment of brain disorders. We are committed to accelerating drug discovery programmes of pharmaceutical companies in psychedelics and neuropsychiatric, neurodegenerative, and rare neurodevelopmental disorders by offering cutting-edge clinical and preclinical research services located at Cherrywood Business Park and Trinity College Dublin in the Republic of Ireland. Our research platforms include therapeutic interventions, access to rare diseased populations, extensive clinical and preclinical molecular biomarker capabilities, in vitro human iPSC rodent and rodent assays, preclinical pharmacological and transgenic rodent models coupled with behavioural and EEG analyses. Guided by our core values of patient-centricity, social responsibility, and a focus on translational research, we are your trusted ally in transforming scientific breakthroughs into tangible treatments.

www.ulysses-neuro.ie/

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/

Partner with the 5th ALS Summit

Tap into a Global Network of Leading CNS Drug Developers, Forge Strategic Partnerships & Propel Your Business Forward

With the ALS and FTD R&D field facing a tumultuous few years of wins and clinical setbacks, the pharma and biotech community remain committed to the field, funding programs with strong biological rationale and long-term potential. Fueled by significant strategic investment, including **Eli Lilly's** $1B+ deployed across multiple ALS partnerships, **Takeda's** ~$580M entry into ALS via **AcuraStem**, and **BMS's** $2B+ collaboration with insitro to uncover genetically validated ALS targets, the ALS community continues to drive towards and cure.

Despite continued investment, research is hindered by bottlenecks spanning disease heterogeneity and model translation to biomarker development, trial execution, and regulatory risk. The **5th ALS Drug Development Summit** brings together 100+ senior leaders, innovators, and scientists actively seeking an edge to advance their research.

Are you looking to elevate your continued commitment to the ALS and FTD community and driving meaningful progress for patients?

> "Great presentations and networking."
> **Senior Manager, Business Development, BrainXell**

> "Great research and key opinion leader availability to discuss."
> **Director, Business Development, Elixirgen Scientific**

> "The conference was very intimate which made it much easier to strike up conversations and interact with presenters."
> **Data Scientist, Unlearn AI**

> "Small but good to meet a lot of people!"
> **Associate Director, Community Education, ALS Therapy Development Institute**

Drug Developers are Actively Seeking:

- ***In Vitro* models:** 3D Cultures, hIPSCs and brain organoids to mimic complex and heterogenous ALS and FTD pathology
- **Clinical CROs:** Offering trial design and program management with strong PI connections, reliability and efficiency
- ***In Vivo* Models:** Including TDP43, and C9orf72 rodent models to better predict efficacy in humans
- **Biomarker Discovery:** Largescale 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

A — Accelerate High-Value Connections & Opportunities

Amplify your exposure to industry leaders through curated networking sessions and 1-to-1 meetings. Forge meaningful relationships that translate into new business opportunities across the ALS landscape.

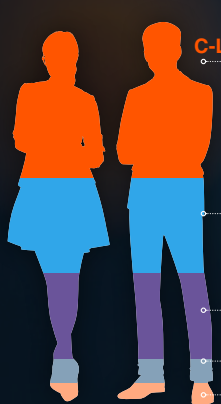
L — Learn from the Latest Market Shifts

Stay informed on emerging trends, innovative therapies, and evolving clinical trial approaches. Gain insights that help you stay ahead of a rapidly changing ALS research and treatment environment.

S — Strengthen Your Product Position & Become a Partner of Choice

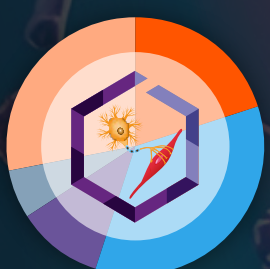
Showcase your unique capabilities and services to position your company as a strategic partner for ALS drug developers. Stand out among providers, imaging specialists, and CROs by demonstrating a strong track record in neurodegenerative disease research and collaboration.

Seniority of Attendees:



- C-Level/VP: 41%
- Director & Head of: 24%
- Manager & Group Leads: 22%
- Scientist: 6%
- Other: 7%

Types of Companies Attending:



- Pharma: 20%
- Biotech: 35%
- Academic, NPO & Investor: 11%
- Patient, Advocates & Caregivers: 6%
- Service Provider: 28%

- **Brand Visibility & Awareness:** On-site and website branding to highlight your company at the forefront of kidney disease research
- **Early Meeting Planning:** Gain exclusive access to the audience delegate list
- **Showcase Your Solutions:** Demonstrate your solutions and capabilities to potential clients with a custom exhibition booth
- **Position Yourself as Thought Leader:** Deliver data-driven or case study-led presentations
- **Customized Partnership:** Our expert team will work closely with you to create a partnership that aligns with your business objectives

Ready to Accelerate Your Business in CNS Drug Development?

Get in touch today to create your bespoke partnership establishing your company as a leading force in the industry and play a pivotal role in bringing better drugs to patients faster.

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

Ready to Register?

3 Easy Ways To Book

www.als-drug-development.com

info@hansonwade.com

+1 617 455 4188

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 100+ 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

Drug Developer Pricing	Register & Pay By Friday, February 20 & Save Up To $1000	On the Door
Conference + Workshop Day	$2,697	$3,697
Conference Only	$2,199	$2,699
Academic Pricing	**Register & Pay By Friday, February 20 & Save Up To $500**	**On the Door**
Conference + Workshop Day	$2,497	$2,997
Conference Only	$1,799	$2,299
Service Provider Pricing	**Register & Pay By Friday, February 20 & Save Up To $1000**	**On the Door**
Conference + Workshop Day	$3,597	$4,597
Conference Only	$2,899	$3,399

*To qualify for the drug developer rate your company must have a public drug pipeline and not offer fee-based services. Please visit the website for full pricing options or email info@hansonwade.com

Do you work for a Not-for-Profit organisation? Email us at info@hansonwade.com to inquire about attending

**To qualify for academic & research rate you must be full time academic. Please visit the website for full pricing options or email info@hansonwade.com

Multi-Pass Discounts

10% discount 2 passes

15% discount 3 passes

20% discount 4+ passes

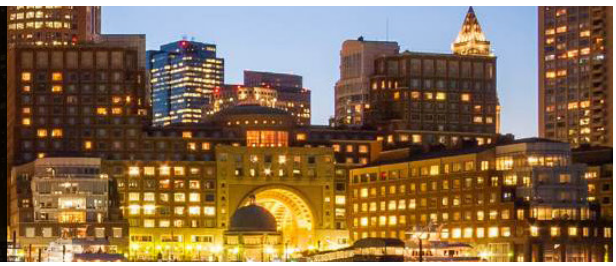
Please note that group discounts are only valid when two or more delegates from one company book and pay at the same time. Only one discount offer may be applied to the current pricing rate. Contact: register@hansonwade.com

VENUE

The Colonnade Hotel

120 Huntington Ave, Boston, MA 02116, United States

www.colonnadehotel.com



Terms & Conditions
Full payment is due on registration.

Cancellation and Substitution Policy:
Cancellations must be received in writing. If the cancellation is received more than 14 days before the conference attendees will receive a full credit to a future conference. Cancellations received 14 days or less (including the fourteenth day) prior to the conference will be liable for the full fee. A substitution from the same organization can be made at any time.

Changes to Conference & Agenda:
Every reasonable effort will be made to adhere to the event programme as advertised. However, it may be necessary to alter the advertised content, speakers, date, timing, format and/or location of the event. We reserve the right to amend or cancel any event at any time. Hanson Wade is not responsible for any loss or damage or costs incurred as a result of substitution, alteration, postponement or cancellation of an event for any reason and including causes beyond its control including without limitation, acts of God, natural disasters, sabotage, accident, trade or industrial disputes, terrorism or hostilities

Data Protection: The personal information shown and/or provided by you will be held in a database. It may be used to keep you up to date with developments in your industry. Sometimes your details may be obtained or made available to third parties for marketing purposes. If you do not wish your details to be used for this purpose, please write to: Hanson Wade, Eastcastle House, 27/28 Eastcastle Street, London, W1W 8DH