February 23-25, 2021 | Digital Event

9AM-7PM EST | 6AM-4PM PST

www.neurodegenerative-drug-development.com

Register & Pay Before November 6 & Save Up to $600

WELCOME

SPEAKERS

AGENDA

PARTNER WITH US

REGISTER YOUR PLACE

9th Annual Neurodegenerative Drug Development Summit

DIGITAL

Meaningfully Translate Innovative Discoveries & Advances in Neurodegenerative R&D to Improve Clinical Efficacy & Patient Outcome

32 Expert Speakers Include:

Martin Tolar
Founder, President & CEO
Alzheon

Gennaro Pagano
Expert Medical Director, Group Leader in Early Development, & Chair of Medical Directors Fellowship
Roche Pharma and Early Development (pRED)

Sanjay Kakka
President & CEO
Tranquis Therapeutics

Michelle Hastings
Director, Center for Genetic Diseases, Professor
Chicago Medical School, RFUMS

Jeff Dage
Senior Research Advisor
Eli Lilly & Company

Michael Kurnellas
Staff Scientist
Alector

José Luis Molinuevo
VP Clinical Development Neurodegeneration
Lundbeck

Elena H Martínez-Lapiscina
Rare Neurological Disorders Lead - Clinical Neurologist
European Medicines Agency

Janna Hutz
Head of Discovery Data Science
Eisai Center for Genetics Guided Dementia Discovery (G2D2)

2021 Partners

atuka | The Jackson Laboratory | CERTARA | NSA

+1 617 455 4188 info@hansonwade.com

www.neurodegenerative-drug-development.com

hansonwade

Welcome to the 9th Neurodegenerative Drug Development Summit

9th Annual Neurodegenerative Drug Development Summit

February 23-25, 2021 | Digital
9AM- 7PM EST | 6AM-4PM PST

Back with a bang, the **Neurodegenerative Drug Development Summit** is the industry's definitive and unrivalled forum focused on revealing hot and promising pockets of innovation in drug discovery for neurodegenerative diseases and combating translational challenges by shining a light on pioneering companies leading the way to meet this dire medical need.

Building on the success of last year's meeting, this year's program showcases new biotechs trailblazing this space and will put the spotlight on the latest scientific advances in terms of **diverse targets, novel modalities** and **innovative trial design**.

Across 3 action-packed, case-study driven days and 2 parallel tracks of learning, we present the opportunity to join us online in 2021 to overcome technical and operational challenges preventing you and your team from translating promising preclinical research into evidenced clinical benefit.

Whether you are working in a team dedicated to **Parkinson's, Alzheimer's, ALS, Frontotemporal Dementia** or a **more rare neurodegenerative disorder**, join this intimate, industry-led forum to not only to learn from those spearheading this space but to network and build meaningful partnerships with over 180 neurodegenerative drug development experts. After all, collaboration in this industry is pivotal.

Mel Melville
Program Director
Neurodegenerative Drug Development Summit

What previous attendees have had to say:

"A very good overview of the state of the art issues that are confronting the field of neurodegeneration"

Brett Skolnick, Executive Director - Clinical Development, **Gossamer Bio**

"High quality, excellent speakers, good networking"

Jan DeWitt, Vice President, **Covance**

Your unique opportunity to:

Discover diverse approaches to regeneration therapeutics with **Aspen Neuroscience** and **Neuronascent**, and explore cellular approaches to neurodegenerative drug discovery with **Noveome**

Hear novel approaches to developing protein misfolding-specific therapies for the treatment of neurodegeneration with **ProMis Neurosciences** and **Origami Therapeutics**

Evaluate pioneering approaches to clinical design for ALS with **Harvard Medical School** and hear how **Roche** are targeting Alpha-Synuclein to design the next-generation of clinical trials

Investigate post-translational modifications as a new therapeutic approach for CNS disorders with **Circumvent Pharmaceuticals** and hear how **Stoke Therapeutics** are developing a unique therapeutic approach to tackle neurodegenerative disorders using antisense oligonucleotides

Explore the implementation of novel blood-based biomarkers, evaluate the role of CNS exosomes in diagnosing AD & PD and assess an encyclopedia of available biomarkers for neurodegenerative diseases with **Zetterberg**, **the EMA**, **Eli Lilly & Company**, **Rissman** and **Teunissen**

Unpack the role that genetics play in neurodegenerative diseases and explore emerging new gene therapies for CNS indications with **Eisai** and **Brain Neurotherapy Bio**

+1 617 455 4188 info@hansonwade.com
www.neurodegenerative-drug-development.com

hansonwade

Your 32 Expert Speakers

Henrik Zetterberg
Professor
University of Gothenburg

Martin Tolar
Founder, President & CEO
Alzheon

Gennaro Pagano
Expert Medical Director, Group Leader in Early Development, and Chair of Medical Directors Fellowship
Roche Pharma and Early Development (pRED)

Sanjay Kakka
President & CEO
Tranquis Therapeutics

Michelle Hastings
Director, Center for Genetic Diseases, Professor
Chicago Medical School, RFUMS

Jeff Dage
Senior Research Advisor
Eli Lilly & Company

Michael Kurnellas
Staff Scientist
Alector

Sabrina Paganoni
Assistant Professor
Harvard Medical School

Elena H Martínez-Lapiscina
Rare Neurological Disorders Lead - Clinical Neurologist
European Medicines Agency

Janna Hutz
Head of Discovery Data Science
Eisai Center for Genetics Guided Dementia Discovery (G2D2)

Dimitry Ofengeim
Precision Neurology & Neuroinflammation Head
Sanofi

Vanessa Almendro
Head of Strategy & Operations
Eisai Center for Genetics Guided Dementia Discovery (G2D2)

Gene Liau
Executive Vice President; Head Research & Preclinical Development
Stoke Therapeutics

Mark Treherne
Chairman
Gen2 Neuroscience

Judith Kelleher-Andersson
President & CEO
Neuronascent

Howard Federoff
CEO
Aspen Neuroscience

Beth Hoffman
Founder, President & CEO
Origami Therapeutics

Michele Vendruscolo
Professor & CSO
Cambridge University Centre for Misfolding Diseases & Wren Therapeutics

Robert Rissman
Professor
UC San Diego

Don W Cleveland
Chair, Distinguished Professor of Cellular & Molecular Medicine & Neurosciences Member
Ludwig Institute for Cancer Research

Jonathan Behr
Partner
Dementia Discovery Fund

Your 32 Expert Speakers

Larry Brown
CSO
Noveome Biotherapeutics

Andrew Lim
CEO
Circumvent Pharmaceuticals

Maurice Zauderer
President & CEO
Vaccinex

Isaac Veinbergs
President & CEO
Libra Therapeutics

Jonathan Levenson
Vice President of Translational Biology
Tiaki Therapeutics

Neil Cashman
CSO of **ProMIS Neurosciences,**
Professor, Neurology
University of British Columbia

Krystof Bankiewicz
President & Chief Executive Officer, **Brain Neurotherapy Bio, Inc,** CEO of **Columbus Children's Foundation**

Selina Wray
Alzheimer's Research UK Senior Research Fellow, Professor of Molecular Neuroscience
UCL

Daniel Lorrain
CSO
Pipeline Therapeutics

José Luis Molinuevo
VP Clinical Development Neurodegeneration,
Lundbeck

Charlotte Teunissen
Professor Neurochemistry
VUmc

"Very collaborative, informative meeting with the right balance between "deep" science and commercial aspects of drug development"

Julian Arbuckle, Chief Business Officer & Co-Founder, **Black Swan Pharmaceuticals**

"Perfect size - large enough to be exposed to many different things and small enough to have in depth conversations with people... Great overview of where the field stands"

Alex Greenfield, Computational Scientist,
Integral Health

"It was a great conference with expert speakers and state-of-the-art science discussion relevant to the neurodegenerative field."

Rajesh Iyengar, Research Fellow, Medicinal Chemistry, **Cyclerion Therapeutics Inc.**

Preconference Workshop Day
Tuesday, February 23

8:30AM-6:00PM EST

Workshop A
8:30-10:30 EST

Leveraging Patient-derived Induced Pluripotent Stem Cells (iPSC) to Model Neurodegenerative Diseases

Research into neurodegeneration has been limited by the lack of pre-clinical *in vitro* and *in vivo* models that fully recapitulate key aspects of disease pathology. This session looks to explore how the availability of induced pluripotent stem cells from patients with phenotypes and genotypes of interest has revolutionised our ability to generate physiologically relevant disease models for neurodegenerative diseases. Here we will learn how we can use these models to elucidate neurodegenerative disease mechanisms and therefore develop targeted therapies.

We will tackle the following topics:
- Evaluating 2D differentiated neurons and organoids as models of neurodegenerative disease, what opportunity and challenges do they present?
- What approaches are there to utilizing each technique for modelling disease, what distinct advantages are there to each new tool?
- To what extent can they be used as a valuable model system in Alzheimer's disease research, and what novel insights into disease mechanisms can be gained from these models?
- How reflective are the phenotypes observed with iPSC of changes in patient biosamples such as CSF, plasma and post-mortem tissue?
- Can we use iPSC to move beyond neuro-centric disease modelling, and create novel multicellular disease models?
- What are the respective advantages/disadvantages of patient cells vs. isogenic ilines engineered using CRISPR?

This workshop will equip you with the knowledge to understand the strengths and limitations of iPSC as pre-clinical models in a broad range of research settings and an appreciation of the considerations to be made before embarking on iPSC research.

Workshop Leader:

Selina Wray
Alzheimer's Research UK Senior Research Fellow, Professor of Molecular Neuroscience
UCL

Workshop B
11:00-13:00 EST

How Best to Design New, Innovative & Cost-Effective Clinical Trials for Neurodegenerative Diseases

This year we have seen a lot of excitement in developing biomarkers for a range of neurodegenerative disorders. This workshop aims to evaluate new fluid biomarkers and how they can be used in clinical trials together with other diagnostic modalities when we have disease modifying drugs on the market.

The session will study concrete examples across the industry and determine how we can speed up clinical trials and the cost savings associated with this approach.

We aim to discuss the following topics:
- What are the gaps in terms of the implementation of blood biomarker panels in clinical routine or clinical trials?
- How should biomarkers be used to design clinical trials? What examples can we learn from or follow?
- How should we use the various biomarkers available across different clinical settings?
- How will biomarkers be used the day we have effective disease-modifying therapies?
- What approaches to leveraging biomarkers to identify subgroups of patients are going to respond best to therapy?
- Combining analytical validation and clinical validation to develop tests with optimal clinical value for a specific context-of-use

Leave this session with a thorough understanding of how to develop fluid biomarkers for clinical validation and implementation for clinical use.

Workshop Leaders:

Henrik Zetterberg
Professor
University of Gothenburg

Charlotte Teunissen
Professor
Neurochemistry
VUmc

Preconference Workshop Day
Tuesday, February 23

8:30AM-6:00PM EST

Workshop C
13:30-15:30 EST

Emerging Gene Delivery Technology for Neurodegenerative Diseases

Using Parkinson's, Multiple System Atrophy and Huntington's as clinical case studies, this session looks to address the following key topics:

- What novel devices are advancing for gene therapy delivery specifically for neurodegenerative diseases?
- What improvements have there been in the last year?
- What common challenges are we yet to have overcome?
- Considering the route of administration and patient population size, to what extent is gene therapy a viable option for Alzheimer's Disease and Parkinson's Disease?
- Advances and progress in developing gene therapies for the rare and ultra-rare neurodegenerative disorders

Workshop Leader:

Krystof Bankiewicz
President & Chief Executive Officer, **Brain Neurotherapy Bio**, CEO of **Columbus Children's Foundation**

Workshop D
16:00-18:00 EST

Exploring Remyelination Promoting Therapies for Neurodegenerative Diseases

This workshop aims to dive into the topic of remyelination and all the critical questions arising around advancing this approach as a therapy for neurodegenerative diseases.

We aim to discuss the following topics:

- Exploring the prospect of licensed remyelinating treatments in MS
- What are the barriers to remyelination in humans?
- What is the process of remyelination and what is the latest research that has given new insights into this process?
- How does remyelination work and what goes wrong?
- How can we develop novel methodologies and medications to promote remyelination and repair of the affected nerve cells?
- How well do the animal models used to research remyelination match the process in humans?
- How can we best translate this into clinical trials?
- What other diseases might be treated with a remyelination approach?
- What tools can we use to best demonstrate that a drug is having an effect by promoting remyelination, what advanced MRI techniques and PET scans are being developed?
- When would be the best time to start a remyelinating treatment?
- What innovative research strategies are being applied to develop novel drugs for the treatment of MS?

Workshop Leader:

Daniel Lorrain
CSO
Pipeline Therapeutics

Conference Day One
Wednesday, February 24

9AM-7PM EST | 6AM-4PM PST

9.00 | 6.00 **Chair's Opening Remarks**

Isaac Veinbergs
President & CEO
Libra Therapeutics

Setting the tone for the meeting: with the abundance of new mechanistic insights coupled with novel tools for design and diverse modalities of target engagement, we are certainly in a position for great optimism and promise in the field

9.10 | 6.10 **Kick-off Panel Discussion:**

- Overview of recent encouraging clinical case studies across the industry – what are the treatments that are around the corner promising results?
- What has Biogen's aducanumab story taught us?
- What new research has been revealed to shine light on the mechanisms that are driving neurodegenerative pathology?
- What are the learnings from a more acute neurological injury like a stroke or a TBI or Guillain-Barre syndrome?
- Is there space for all of these different modalities out there? Where should we concentrate our efforts?

10.00 | 7.00 **Speed Networking**

This session is the ideal opportunity to get face-to-face time (albeit 2D faces) with many of the brightest minds working towards in neurodegenerative drug development. Benchmark against the industry leaders and establish meaningful business relationships to pursue for the rest of the conference and beyond.

Discovery Stream

Evaluating Monogenic Diseases, Protein Misfolding & Toxic Tau

11.00 | 8.00 New Targets & Approaches to Targeting Monogenic Neurodegenerative Diseases

- What insights are we gaining from studying monogenic diseases to understand the complexity of much broader diseases?
- How can we leverage antisense oligonucleotide technology to find new targets for monogenetic diseases?
- Exploring the therapeutic efficacy of antisense oligonucleotides in mouse models of CLN3 Batten disease
- What is the clinical progress in rare monogenic neurodegenerative conditions?
- Broadening the therapeutic landscape for ASOs in the treatment of neurodegenerative diseases

Michelle Hastings, Director, Center for Genetic Diseases, Professor, **Chicago Medical School, RFUMS**

11.30 | 8.30 Precision Immunotherapies for Protein Misfolding Diseases to Advance ALS & FTD Therapeutics

- Exploring how the ProMIS Platform is being leveraged to develop protein misfolding-specific immunotherapies for neurodegeneration
- Case study: selective targeting of toxic forms of TDP-43 in ALS and FTD, while sparing normally folded functional TDP-43
- Dissecting the role of prion-like propagation of misfolded proteins in neurodegeneration

Neil Cashman, CSO of **ProMIS Neurosciences**, Professor, Neurology, **University of British Columbia**

Translational Stream

Driving Innovation in Clinical Trial Design

11.00 | 8.00 Lessons Learned From Clinical Trials Targeting Alpha-Synuclein to Design Next-Generation Clinical Trials

- How the results of the Phase 2 PASADENA study of Prasinezumab, a humanized monoclonal antibody for Parkinson's disease, can inform future clinical trials
- How to apply a precision medicine approach in Parkinson's disease clinical trials?
- Can we predict cognitive decline in Parkinson's Disease to design studies evaluating this endpoint (lesson learned from PRECODE studies)?

Gennaro Pagano, Expert Medical Director, Group Leader in Early Development, & Chair of Medical Directors Fellowship, **Roche Pharma and Early Development (pRED)**

11.30 | 8.30 Pioneering Innovative Trial Design to Develop New Treatments for ALS

- How to design more efficient and more effective clinical trials and broaden access for patients
- Key considerations when choosing novel endpoints and biomarkers into innovative trial designs
- Identifying and working with multiple stakeholders
- Diving into the HEALEY ALS Platform Trial

Sabrina Paganoni, Assistant Professor, **Harvard Medical School**

Conference Day One
Wednesday, February 24
9AM-7PM EST | 6AM-4PM PST

12.00 | 9.00 Intraneuronal Transmission of Toxic Tau Species

- There are multiple species of tau but specific toxic species are taken up by neurons
- Others species of tau have different properties but also bind potential therapeutic antibodies
- Antibodies that selectively bind to the toxic tau species have the potential to be the next generation of therapeutics for the treatment of Alzheimer's Disease

Mark Treherne, Chairman, **Gen2 Neuroscience**

12.00 | 9.00 Reverse Translation in Alzheimer's Disease

- Reverse translation allows hypothesizing on additional pathological pathways, on their "timing" along the continuum and gives an idea of the potential effect size of an intervention
- APOE4 not only increases the risk of amyloid deposition but also generates structural vulnerability
- New CSF biomarkers are showing early physiopathological changes in multiple biological cascades
- It is key to define WHEN to intervene as a function of the compound MoA for a successful proof of concept
- Fluid and imaging biomarkers allow us understanding the role of genetic and environmental risk factor

José Luis Molinuevo, VP Clinical Development , Neurodegeneration, **Lundbeck**

12.30 | 9.30 Live Q&A With Your Expert Speakers

Mark Treherne, Chairman, **Gen2 Neuroscience**

Michelle Hastings, Director, Center for Genetic Diseases, Professor, **Chicago Medical School, RFUMS**

Neil Cashman, CSO of ProMIS Neurosciences, Professor, Neurology, **University of British Columbia**

12.30 | 9.30 Live Q&A With Your Expert Speakers

Sabrina Paganoni, Assistant Professor, **Harvard Medical School**

José Luis Molinuevo, VP Clinical Development , Neurodegeneration, **Lundbeck**

Gennaro Pagano, Expert Medical Director, Group Leader in Early Development, & Chair of Medical Directors Fellowship, **Roche Pharma and Early Development (pRED)**

13.00 | 10.00 Lunch Break

14.00 | 11.00 Targeting Myeloid Immune Cell Dysfunction to Revolutionize Neurodegenerative Treatment – From Discovery to the Clinic

Sanjay Kakka
President & CEO
Tranquis Therapeutics

- Targeting a master metabolic pathway that is downregulated in myeloid cells in patients with neurodegenerative diseases
- Identifying our lead compound and designing our candidates to reduce inflammation in dysfunctional myeloid cells and reprogram them to restore their normal immuno-metabolic function
- Data demonstrating highly encouraging in vivo effects in challenging preclinical models of neurodegenerative diseases
- Demonstrating a clear link between mechanisms, phenotype, models and reduction in inflamed myeloid cells
- Exploring our clinical biomarker that strengthens our proof of concept
- Patient studies to establish dosage vs kinetics and effect of target cell type
- Outlining plans to initiate clinical development in 2021 for orphan diseases such as ALS and FTD and later PD and AD

14.30 | 11.30 Targeting Amyloid Beta for Alzheimer's Disease

Michele Vendruscolo
Professor & CSO
Cambridge University Centre for Misfolding Diseases & Wren Therapeutics

- Amyloid beta is a primary target for Alzheimer's Disease
- Amyloid beta is the reactant, product, intermediate and catalyst in the aggregation reaction
- Choosing the right conformational state of amyloid beta is critical for therapeutic efficacy
- Advances in measuring the thermodynamics and kinetics of aggregation is revealing target engagement
- Based on these advances, the possibility of effective treatments is emerging

Conference Day One
Wednesday, February 24
9AM-7PM EST | 6AM-4PM PST

15.00 | 12.00 **ALZ-801 Phase 3 Program: A Targeted Oral Disease Modifying Therapy for Alzheimer's Disease**

Martin Tolar
MD, PhD, Founder, President & CEO
Alzheon

- Convergence of scientific evidence that soluble amyloid oligomers are directly neurotoxic upstream drivers of Alzheimer's disease pathology
- Agents targeting amyloid oligomers show positive clinical and biomarker effects in Alzheimer's patients treated with Alzheon's ALZ-801, as well as anti-amyloid antibodies BAN2401 and aducanumab
- Alzheon's oral tablet ALZ-801 acts upstream in the disease process to fully block formation of amyloid oligomers, with favorable safety and tolerability, opening the possibility of preventive treatment
- Alzheon's applies precision medicine to initially focus on Alzheimer's patients homozygous for apolipoprotein E4 allele, who have increased brain levels of amyloid oligomers, and are the target population for FDA Fast Tracked Phase 3 trial supported by a $47M NIH grant award
- Integration of leading-edge disease modification biomarkers into ALZ-801 drug development increases confidence in a successful outcome

15.30 | 12.30 **Clinical Case Study: Exploring AL001 for the Treatment of FTD**

Michael Kurnellas
Staff Scientist
Alector

- AL001 is an anti-Sortilin antibody that inhibits progranulin (PGRN) binding and lysosomal degradation, thereby raising the levels of PGRN
- An increase in PGRN was observed in the CSF with target engagement in Grn-deficient mice
- Interim data from the Phase 2 clinical trial shows that AL001 was generally safe and well tolerated and increased the levels of PGRN back to the normal range in participants with frontotemporal dementia (FTD) with heterozygous GRN mutations

16.00 | 13.00 **Live Q&A With Your Expert Speakers**

Martin Tolar
MD, PhD, Founder, President & CEO
Alzheon

Sanjay Kakka
President & CEO
Tranquis Therapeutics

Michele Vendruscolo
Professor & CSO
Cambridge University Centre for Misfolding Diseases & Wren Therapeutics

Michael Kurnellas
Staff Scientist
Alector

16.30 | 13.30 **Networking Break**

Discovery Stream

Advancing New Approaches to Drug Discovery

16.50 | 13.50 Exploring Cellular Approaches to Neurodegenerative Drug Discovery

- Origin of Amnion-derived Multipotent Progenitor (AMP) Cells
- Stem-cell like properties of AMP cells
- Neuroprotective and immunomodulatory activities of AMP cells
- AMP cell secretome

Larry R. Brown, Executive Vice President, Chief Scientific Officer, **Noveome Biotherapeutics**

Translational Stream

Exploring Regenerative Therapy & Translational Tools

16.50 | 13.50 Pioneering Neuron Regeneration Therapeutics to Fill the Void in Treatment Options for AD

- Exploring the unique mechanism of action of NNI-362, selectively stimulating the conversion of human brain neural progenitor cells to mature functioning neurons
- Allowing the function to guide the target, assessing the journey from phenotypic screening of regenerative targets to the clinic
- Safety profile in aged humans (SAD)
- Plasma biomarkers can be used post-Phase 1a to guide the future POC trial
- Evaluating brain adult-born neurons numbers in future trials based on preclinical data in aged and Down syndrome-modeled animals

Judith Kelleher-Andersson, President & CEO, **Neuronascent**

Conference Day One
Wednesday, February 24
9AM-7PM EST | 6AM-4PM PST

17.20 | 14.20 Investigating Post Translational Modifications as a New Therapeutic Approach for CNS Disorders

- Unravelling a new area of biology to help elucidate the functional relationship between certain PTMs and protein localization
- Mapping the palmitoylome and palmitoylation gene interactome yields putative disease targets
- Hypotheses of how dysregulation of palmitoylation and protein mislocalization drives disease
- Developing a novel class of molecules to target this area, focusing on Batten Disease and leveraging these insights to drive further understanding of AD

Andrew Lim, Chief Executive Officer, **Circumvent Pharmaceuticals**

17.20 | 14.20 Leveraging Stem Cell Biology & Genomics for Regenerative Therapy for Parkinsons

- Providing a personalized cell therapy that avoids the need for immunosuppression using Autologous Induced Pluripotent Stem Cell-Derived Neurons
- Comparison of autologous and allogenic therapy and reviewing allogenic approaches across the neurologic indication space
- iPSCs have all the strengths of embryonic stem cells without the limitations of allogeneic therapy
- Innovation in clinical development and leveraging a machine learning approach for cellular identify and purity with regulatory implications

Howard Federoff, Chief Executive Officer, **Aspen Neuroscience**

17.50 | 14.50 Discovery of Small Molecules for the Treatment of Neurodegeneration Caused by Protein Misfolding: Huntington's Disease as a Case Study

- Phenotypic screening to identify protein conformation modulators: discovery of protein degraders and conformation correctors
- Characterization of small molecules using patient-derived disease models and machine learning to prioritize small molecules based on efficacy and safety
- Why we chose to tackle Huntington's disease and how we can leverage our learnings to approach other neurodegenerative diseases

Beth Hoffman, Founder, President & CEO, **Origami Therapeutics**

17.50 | 14.50 An Integrated Platform for Identification and Validation of Targets for Mitigation of Neuroinflammation in Neurodegenerative Diseases

- Exploring how our systems biology platform is used to reveal novel targets for dementias
- Linking human disease datasets with *ex vivo / in vivo* models of neuroinflammation
- Identifying disease biology across 5 neurodegenerative diseases
- How can we best validate neuroinflammatory targets identified in patients?
- Discovering the best translational tools and models for studying neuroinflammation

Jonathan Levenson, Vice President of Translational Biology, **Tiaki Therapeutics**

18.20 | 15.20 Live Q&A With Your Expert Speakers

Larry R. Brown, Executive Vice President, Chief Scientific Officer, **Noveome Biotherapeutics, Inc.**

Andrew Lim, Chief Executive Officer, **Circumvent Pharmaceuticals**

Beth Hoffman, Founder, President & CEO, **Origami Therapeutics**

18.20 | 15.20 Live Q&A With Your Expert Speakers

Howard Federoff, Chief Executive Officer, **Aspen Neuroscience**

Judith Kelleher-Andersson, President & CEO, **Neuronascent**

Jonathan Levenson, Vice President of Translational Biology, **Tiaki Therapeutics**

18.40 | 15.40 Highlights From Both the Discovery & Translational Streams

If you missed half of the translational stream, do not fear! This session will collate all the key learnings from across the two streams into one stellar quick fire summary. A perfect way to end the day!

18.50 | 15.50 Chair's Closing Remarks

Conference Day Two

Thursday, February 25

9AM-7PM EST | 6AM-4PM PST

Isaac Veinbergs
President & CEO
Libra Therapeutics

9.00 | 6.00 **Chair's Opening Remarks**

9.10 | 6.10 **Driving Investment in Neurodegeneration**

- Exploring the novel therapeutics and mechanisms in drug discovery that are attracting investment
- What is the interplay between large markets / unmet medical need and smaller indications with better defined patient populations?
- How are companies overcoming the historical challenges in neurodegenerative drug discovery and attracting investment?

Jonathan Behr
Partner
Dementia Discovery Fund

Discovery Stream

Diving Into Human Genetics, Target Identification & Neuroinflammation

9.40 | 6.40 Unpacking the Role that Genetics Play in Neurodegenerative Diseases

- Leveraging insights from human genetics, computational biology, and other data sciences to drive and maximise discovery programs
- Exploring case studies of progressing genetically validated targets through discovery and development
- Assessing different modalities and matching the target to the right modality using human genetics
- Using genetics to identify the right biomarker and target patient population

Janna Hutz, Head of Discovery Data Science, **Eisai Center for Genetics Guided Dementia Discovery (G2D2)**

10.10 | 7.10 Modern Target Identification: Exploration of the Hypotheses Around the Use of Genetics to Inform Decision Making

- What does it mean for a target to be genetically enabled?
- How should we use genetics and genomics data to identify and prioritize drug targets for therapeutic development?
- What is the role of preclinical tools if human genetic data are the primary driver for target selection?

Janna Hutz, Head of Discovery Data Science, **Eisai Center for Genetics Guided Dementia Discovery (G2D2)**

Jonathan Levenson, Vice President of Translational Biology, **Tiaki Therapeutics**

10.40 | 7.40 Delving Into the Interface of Neuroinflammation & Neurodegeneration

- Exploring the mechanisms of action for RIPK1-medicated neuroinflammation in CNS diseases, focusing on ALS,MS and beyond.
- Reviewing RIPK1 biology and disease-associated mutations in RIPK1 signalling pathways
- Pioneering applications of these RIPK1 inhibitors for the treatment of monogenic and polygenic neurodegenerative disorders
- Early discovery work underway for future endeavours

Dimitry Ofengeim, Precision Neurology and Neuroinflammation Head, **Sanofi**

11.20 | 8.20 Live Q&A With Your Expert Speakers

Dimitry Ofengeim, Precision Neurology & Neuroinflammation Head, **Sanofi**

Janna Hutz, Head of Discovery Data Science, **Eisai Center for Genetics Guided Dementia Discovery (G2D2)**

Translational Stream

Advancing the Application of Novel Biomarkers Into Clinical Practic

9.40 | 6.40 An Encyclopaedia of Currently Available Biomarkers for Neurodegenerative Diseases

- Dissecting the current state of AD research, diving into new therapeutics and biomarkers
- What do we have now and what needs to be developed?
- How about biomarkers for non-AD neurodegenerative diseases?
- What is needed for implementation of new biomarkers in clinical practice?
- How can biomarkers be used today?
- How will biomarkers be used the day we have effective disease-modifying therapy?

Henrik Zetterberg, Professor, **University of Gothenburg**

10.10 | 7.10 Exploring the Utility of P-tau as a Blood-Based Biomarker in Neurodegeneration

- The science and background of P-tau217 and P-tau181
- Utility of P-tau in diagnosis and prognosis
- Application of P-tau in clinical trials
- Future opportunities that may alter our understanding of Alzheimer's Disease

Jeffrey Dage, Research Fellow, **Eli Lilly & Company**

10.40 | 7.40 Evaluating the Role of CNS Exosomes in Diagnosing AD & PD

- Delving into our approach to using biobanked clinical trials samples, human postmortem tissues and animal models
- Purifying the source of proteins coming from the brain to track progression of neurodegenerative disease
- Exosome trafficking and potential
- Methodological considerations in exosome harvesting

Robert Rissman, Professor, **UC San Diego**

11.20 | 8.20 Live Q&A With Your Expert Speakers

Robert Rissman, Professor, **UC San Diego**

Jeffrey Dage, Research Fellow, **Eli Lilly & Company**

Henrik Zetterberg, Professor, **University of Gothenburg**

11.40 | 8.40 **Networking Break**

Conference Day Two
Thursday, February 25

9AM-7PM EST | 6AM-4PM PST

12.10 | 9.10 **Panel Discussion: Digital Transformation in Neurodegenerative Drug Development**

- How can we implement data science and digital technologies into our clinical trials?
- Digital diagnostic exploration, development of passive data collection for movement disorder symptoms
- Accelerated enrolment solutions and decentralised trials
- Translating trials with cognitive endpoints to a digital format
- Finding and validating digital solutions to cognitive trials, digital biomarkers, digital translation, home assessment
- How can we implement digital technology from a target discovery perspective?
- How can we use artificial intelligent to mine all these databases to find the right targets and monitor and capture real world evidence from patients?
- Can we develop digital technologies that are sensitive enough to be able to diagnose earlier stages of a disease and be applicable broadly?

12.40 | 9.40 **Lunch Break**

Discovery Stream

Delving Into Gene Therapy: How Can We Best Implement New Clinical Trials?

13.30 | 10.30 Emerging New Gene Therapies for CNS Indications

- What is the current status for the emerging new Gene Therapies for CNS indications?
- Choosing the right modality for the right target and the right patient
- Understanding implications in the clinical implementation of Gene Therapies for CNS disorders

Vanessa Almendro, Head of Strategy & External Innovation, **Eisai Center for Genetics Guided Dementia Discovery (G2D2), Eisai**

13.50 | 10.50 Designer DNA Drug Therapy for Neurodegenerative Diseases

- Uncovering the mechanisms underlying the major genetic forms of ALS
- Evaluating ongoing efforts and clinical trials in gene therapy for ALS, FTD, Huntington's Disease, PD and beyond
- Paving the path forward, what's on the horizon?

Don W Cleveland, Chair, Distinguished Professor of Cellular & Molecular Medicine & Neurosciences Member, **Ludwig Institute for Cancer Research**

Translational Stream

Striving for Clinical Validation of Biomarkers

13.30 | 10.30 Assessing Why Preanalytical Testing is Key in Performing High Quality Biomarker Studies

- Cutting-edge developments in blood-based biomarkers for neurodegenerative diseases
- Biomarker identification using proteomics methods, including mass spectrometry and array-based proteomics technologies
- Analytical and clinical validation for diagnostic use of the most promising biomarkers by immunoassays
- Ensuring the quality of biosamples

Charlotte Teunissen, Professor in Neurochemistry, **VUmc**

13.50 | 10.50 Exploring a Regulatory Perspective on Advancing Biomarkers for Neurodegenerative Diseases

- Overview of the potential use of biomarkers for drug development for neurodegenerative conditions
- Experience on EMA Qualification of novel methodologies (biomarkers)
- Genetic markers for neurodegenerative conditions
- Evaluating approaches of using biomarkers for enrichment purposes in clinical trials
- Where does the field need to put more efforts to develop biomarkers that will be able to help us test new hypothesis across neurodegenerative diseases?
- What is the regulatory willingness to accept such biomarkers, what do we need to see in order to validate biomarker endpoint as a primary endpoint for an approval as opposed to a clinical effect?

Elena H Martínez-Lapiscina, Rare Neurological Disorders Lead - Clinical Neurologist, **European Medicines Agency**

Conference Day Two
Thursday, February 25

9AM-7PM EST | 6AM-4PM PST

14.20 | 11.20 Stoking Protein Production – Pioneering a Unique Therapeutic Approach Using Antisense Oligonucleotides

- Designing a platform to address protein deficiency by precisely upregulating target protein expression
- Targeted Augmentation of Nuclear Gene Output (TANGO) technology platform is being used to pursue disease-modifying treatment for severe genetic diseases via a precision medicine approach
- Building data to the clinic for genetic epilepsies and advancing early programs focused on multiple therapeutics areas with initial emphasis on the CNS and the eye
- How might our technology be applicable for neurodegeneration?

Gene Liau, Executive Vice President; Head Research & Preclinical Development, **Stoke Therapeutics**

14.20 | 11.20 Evaluating Clinical Observations & Key Learnings From SIGNAL HD Clinical Trial

- Neurons upregulate SEMA4D in response to stress during neurodegenerative disease progression
- SEMA4D triggers inflammatory activation of astrocytes in HD and AD
- Plans to execute SIGNAL AD in 2021, study design and patient recruitment

Maurice Zauderer, President & CEO, **Vaccinex**

14.50 | 11.50 Live Q&A With Your Expert Speakers

Gene Liau, Executive Vice President; Head Research & Preclinical Development, **Stoke Therapeutics**

Don W Cleveland, Chair, Distinguished Professor of Cellular & Molecular Medicine & Neurosciences Member, **Ludwig Institute for Cancer Research**

Vanessa Almendro, Head of Strategy & External Innovation, **Eisai Center for Genetics Guided Dementia Discovery (G2D2), Eisai US**

14.50 | 11.50 Live Q&A With Your Expert Speakers

Elena H Martínez-Lapiscina, Rare Neurological Disorders Lead - Clinical Neurologist, **European Medicines Agency**

Maurice Zauderer, President & CEO, **Vaccinex**

Charlotte Teunissen, Professor in Neurochemistry, **VUmc**

15.20 | 12.20 Networking Break

15.40 | 12.40 Poster Session

You will have the opportunity to present and review presentations displaying new data from neurodegenerative disease research, such as novel targets, progress with different therapeutic modalities and advances in translational tools.

16.10 | 13.10 Quick-Fire Poster Presentation Talks

Capturing the scientific highlights of the Poster Session, here we will put the spotlight on four exceptional posters

16.30 | 13.30 Wrap Up Panel Discussion: What Have We Learned From the Neurodegenerative Drug Development Meeting?

- Setting future goals for neurodegenerative drug development
- Highlighting the opportunity for drug developers and the hope for patients

17.00 | 14.00 Chair's Closing Remarks & Final Reflections

An Interactive Online Experience

9th Annual Neurodegenerative Drug Development Summit

February 23-25, 2021 | Digital
9AM- 7PM EST | 6AM-4PM PST

The **9th Neurodegenerative Drug Development Summit** is committed to delivering the **high-quality insights** and **industry connections** that our customers expect, in a format that is accessible from the comfort of your home or office.

We have created the virtual summit to satisfy the industry's need to share **cutting-edge research**, learn from your peers and **engage in quality networking** within a niche and highly selective audience to **forge valuable collaborations.**

To effectively facilitate this need to learn and connect, our custom-built virtual event platform will combine best-in-class platforms to deliver a seamless event experience. Accessing the platform is simple, you'll be provided with a unique link in the run up to the event that will take you directly to the online event space where you'll follow a few simple steps to set up your delegate profile and get started.

Key Features & Functionalities:

Delegate Profile
Set up personalized profiles to easily identify the name, job title & company of other attendees

Stage Area
Most presentations will be delivered in the 'Stage' area, much like the main conference room onsite

Sessions Area
Smaller groups can get together in this breakout area for panel discussions and other interactive sessions

Demo Area
Visit the virtual exhibition area to explore the solutions our specialist vendors have on offer

Chat Rooms
Conect with your peers and start conversations with individuals or all attendees in private and public chatrooms

Speed Networking
This virtual networking session will connect you with other attendees to establish new industry contacts

What You Can Expect From a Digital Event:

Live Q&As with Speakers
Ask your burning questions directly to our expert speakers in real-time, just as you would at a physical conference

Audience Discussions
Join smaller, informal group chats or video calls designed to spark crucial conversations around key challenges for the industry

5+ Hours of Networking
Facilitated and informal networking breaks will allow you to connect 1-2-1 with other attendees and kick start critical discussions

Content Available Post-Event
On conclusion of the event, presentations will be made available to attendees where possible

If you have any other questions about the platform, please **get in touch**

+1 617 455 4188 info@hansonwade.com
www.neurodegenerative-drug-development.com

hansonwade

Partnership Opportunities

The **Neurodegenerative Drug Development Summit** will host over 150 leading neurodegeneration experts seeking solutions to help them to confidently translate into the clinic and develop successful neurodegenerative therapeutics.

Showcase your preclinical services, advanced neurodegenerative preclinical models, biomarker innovations, and AI and digital capabilities to this unique industry audience to **outcompete your competitors and fulfill your 2021 business goals.**

There are a variety of partnership opportunities at the summit, including speaking positions, brand exposure and sponsored networking sessions. Please get in touch with us so we can start to build a bespoke partnership package with you to maximize your 2021 business potential.

Partner with the Neurodegenerative Drug Development Summit to **demonstrate your ability to exceed expectations to leading minds in CNS drug development** in the following areas:

- Reliable and high-quality *in vivo* models of neurodegeneration accompanied with high level scientific advice to improve clinical predictions
- The latest *in vitro* models of neurodegeneration including IPSC cells and 3D organoid models
- Exciting fluid biomarker technology for successful integration into clinical trials
- The latest in neuroimaging, from functional PET to neurofilament light advances
- Evidence that your preclinical CRO services are reliable, effective and expertise which would out-do in-house research
- State-of-the-art AI and machine learning technology to transform target identification and data analysis

We will be working with a limited number of select service providers at the **Neurodegenerative Drug Development Summit**. Please contact us to find out more about the partnership opportunities available.

Get Involved

Flavia Alexandru
Partnerships Director
Tel: (+1) 6174554188
Email: sponsor@hansonwade.com

2020 Partners

The **Neurodegenerative Drug Development Summit** is Proud to Partner With:

Expertise Partner:

Atuka provides contract research and consultancy services for the biopharmaceutical industry with world-leading expertise in Parkinson's disease and related neurological conditions. We provide cuttingedge, rodent and non-human primate models (toxin and molecular pathology-driven) to evaluate efficacy and target engagement over a comprehensive range of symptomatic, motor (e.g. parkinsonism and dyskinesia), non-motor (e.g. cognition and impulse control) and disease-modification assays. Atuka also offers medicinal chemistry, DMPK and in-vivo imaging services to aid development of novel therapeutics.

www.atuka.com

Expertise Partner:

The Jackson Laboratory (JAX) is an independent, nonprofit biomedical research institute combining innovation, reliability, and commitment to customer service to provide the most clinically relevant mouse models and precision services. With an extensive and ever expanding portfolio of mouse models and services such as target validation and drug efficacy studies, JAX is committed to providing researchers with the most advanced tools to support their neurobiological research and enable efficacious drug design.

www.jax.org/jax-mice-and-services

Expertise Partner:

Certara is the global leader in advancing modern, efficient drug development. We provide proven modeling & simulation, regulatory and real world value assessment software and services. In partnership with our clients, we help reduce clinical trial burden, accelerate regulatory approval and increase patient access to medicines. 90% of all novel drugs approved by FDA have used Certara software and/ or services.

www.certara.com

Program Partner:

Proprietary MultiBrain® technology powers NSA's simultaneous sectioning, and expertly-guided staining, of up to 40 neuronal tissues in one MultiBrain® block, achieving uniform processing across all 40 tissues. Implementing NSA's Large Format™ technology enables sectioning and staining of intact human brain hemispheres and yields comprehensive, uninterrupted vantage and analysis of the ultimate target. Coupling these mass production technologies with cuttingedge digital image capture and hosting, along with contemporary analysis tools increasingly powered by AI, rapidly produces rich data, and dramatically reduces R&D times.

www.NSALabs.com

Get Involved

Flavia Alexandru
Partnerships Director
Tel: (+1) 6174554188
Email: sponsor@hansonwade.com

Ready to Register?

9th Annual Neurodegenerative Drug Development Summit

February 23-25, 2021 | Digital
9AM- 7PM EST | 6AM-4PM PST

3 Easy Ways To Book

www.neurodegenerative-drug-development.com/take-part/register/

Tel: +1 617 455 4188

Email: register@hansonwade.com

1. **Gain** an important market update on the progress of neurodegenerative drug discovery and development through pioneering case studies in FTD, AD, PD, HD, ALS and more
2. **Discover** novel targets, optimized approaches and technology advances to accelerate the success of your neurodegenerative candidate
3. **Establish** lasting connections with this dedicated community to share, learn and connect for future collaborations to accelerate your products to market and help patients in need

Secure Your Place Now

Academic & Start-Ups	Register & Pay by November 6	Standard Pricing
Conference + 4 Workshops	$1,495 **(Save $800)**	$1,895 **(Save $400)**
Conference + 3 Workshops	$1,296 **(Save $700)**	$1,696 **(Save $300)**
Conference + 2 Workshops	$1,097 **(Save $600)**	$1,497 **(Save $200)**
Conference + 1 Workshop	$898 **(Save $500)**	$1,298 **(Save $100)**
Conference Only	$699 **(Save $400)**	$1,099
Workshops (Each)	$299	

Drug Developer	Register & Pay by November 6	Standard Pricing
Conference + 4 Workshops	$2,595 **(Save $800)**	$2,995 **(Save $400)**
Conference + 3 Workshops	$2,296 **(Save $700)**	$2,696 **(Save $300)**
Conference + 2 Workshops	$1,997 **(Save $600)**	$2,397 **(Save $200)**
Conference + 1 Workshop	$1,698 **(Save $500)**	$2,098 **(Save $100)**
Conference Only	$1,399 **(Save $400)**	$1,799
Workshops (Each)	$399	

Solution Provider	Register & Pay by November 6	Standard Pricing
Conference + 4 Workshops	$3,195 **(Save $800)**	**$3,595 (Save $400)**
Conference + 3 Workshops	$2,896 **(Save $700)**	**$3,296 (Save $300)**
Conference + 2 Workshops	$2,597 **(Save $600)**	**$2,997 (Save $200)**
Conference + 1 Workshop	$2,298 **(Save $500)**	**$2,698 (Save $100)**
Conference Only	$1,999 **(Save $400)**	$2,099 **(Save $300)**
Workshops (Each)	$399	

All prices shown in USD. To qualify for the drug developer rate your company must have a public drug pipeline. To qualify for academic pricing you must have a valid email address for an academic organization. Start-ups must be less than 3 years old or have less than 50 employees. Bookings are subject to the organizer's approval and we reserve the right to refuse bookings made at the incorrect price.

If you are a UK or EU-based company, you may be subject to 20% VAT in addition to the price advertised. If you qualify for a reverse charge, you will have the option to provide your VAT number and the charge will be automatically deducted at checkout.

Please visit the website for full pricing options or email info@hansonwade.com

Team Discounts*

- **10% discount – 3 Attendees**
- **15% discount – 4 Attendees**
- **20% discount – 5 or more Attendees**

*Please note that discounts are only valid when three or more delegates from one company book and pay at the same time.

Discounts cannot be used in conjunction with any other offer or discount. Only one discount offer may be applied to the current pricing rate.

Contact: register@hansonwade.com