February 20–22, 2018 | Boston, MA
www.world-cns.com

6th World CNS Summit
Targeting Neurodegenerative Diseases

Transforming Discovery & Translational Research Into Disease Modifying Therapeutics

29 World-Class Experts Including:

- Tarek Samad
  Head, Neurodegeneration
  Pfizer

- Holly Soares
  Head Translational Neuroscience
  AbbVie

- Johan Luthman
  Vice President, Neuroscience Business Group
  Eisai Pharmaceuticals

- Richard Ransohoff
  Vice President & Senior Research Fellow – Neuroimmunology
  Biogen

- Martin Tolar
  Founder, President & CEO
  Alzheon, Inc.

- Susan Browne
  Director
  Teva Pharmaceuticals

- Alice Zhang
  CEO
  Verge Genomics

- S. Pablo Sardi
  R&D Director
  Sanofi-Genzyme

- Damian Crowther
  Director, R&D Neuroscience IMED Biotech Unit
  AstraZeneca

Expertise Partners:
Welcome to the 6th World CNS Summit: Targeting Neurodegeneration

Advancing Translational Neuroscience For Evidenced Clinical Benefits

The last 12 months have seen the neurodegenerative community make numerous strides into understanding these disorders. However, 2017 has been a rocky journey, particularly so for Alzheimer’s Disease, adding weight to the growing concern as to whether there is a fundamental flaw in our understanding of the disease, its mechanisms or our path to targeting it.

As Program Director & Researcher for the 6th Annual World CNS Summit, I invite you to join a truly diverse group of professionals from biopharmaceutical, technology and academic institutions, in paving the path forward for neurodegenerative therapeutics.

This year we have refined the theme of the meeting to ‘transforming discovery and translational research into disease modifying therapeutics’ to exemplify the vast opportunities that remain at our fingertips.

Focused specifically on the translational barriers in neurodegenerative R&D, this is an opportunity to look beyond A-Beta Tau the and other classical targets of neurodegeneration. It will highlight foundational research into promising targets, such as neuroinflammation, and the strategic approaches to modulate these.

Join over 60 biopharmaceutical organizations and 100 experts in harnessing new innovations, to tackle the challenges at the crux of your pipeline. Redefine the use of preclinical models of neurodegeneration, enhance their application in your translational research and advance translational biomarker discovery - from target engagement to disease progression.

Adam Cohen
Conference Producer & Researcher
World CNS Series

What’s New for This Year?

1. Greater focus on alternative aetiologies of disease including neuro-inflammation, microglial activation and lysosomal dysfunction
2. Increased attention on R&D technologies enabling effective identification of novel targets and candidate therapeutics
3. More preclinical case studies highlighting advances in the translatable of pre-clinical models
4. Strategies to validate clinically relevant biomarkers to inform translational research programmes and improve the success of clinical studies
5. Greater exploration of future therapeutics and strategies beyond the one target, one drug paradigm and Abeta Tau

What Previous Attendees Had To Say:

I liked the breadth of commercial development (pre-clinical up through clinical) included in the program. The meeting is a must go for anyone seriously considering drug development.

St. Lawrence University

I was impressed by many of the speakers and learnt a significant amount, which I was able to bring back to our discovery team.

Stephen Krause, Senior Principal Scientist, Eisai

Excellent networking and learning opportunity for pharma scientists

Matthew Kennedy, Director, Merck Neuroscience

St. Lawrence University

Excellent networking and learning opportunity for pharma scientists

Matthew Kennedy, Director, Merck Neuroscience
Your Expert Speakers

Alice Zhang  
CEO  
Verge Genomics

Brad Margus  
Co-founder & CEO  
Cerevance

Brendon Boot  
Medical Director  
Voyager Therapeutics

Damian Crowther  
Director, R&D  
Neuroscience IMED Biotech Unit  
AstraZeneca

Douglas Bonhaus  
CSO  
Neuropore Therapeutics

Douglas Galasko  
Professor, Department of Neurosciences  
University of California, San Diego

Holly Soares  
Head Translational Neuroscience  
AbbVie

Jan Torleif Pedersen  
Director, TBL Alzheimers Disease and Dementia, Neurodegeneration  
H. Lundbeck A/S

Jay Schneider  
Professor, Pathology, Anatomy and Cell Biology, Director, Parkinson’s Disease Research Unit  
Thomas Jefferson University

Jeffrey Cummings  
Director  
Cleveland Clinic

Johan Luthman  
Vice President, Neuroscience Business Group  
Eisai Pharmaceuticals

Jonathan Brotchie  
Co-Founder and Chairman  
Atuka Inc.

Jonathan Levenson  
Senior Director, Preclinical Research & Development  
Proclara Biosciences

Krzysztof Wicher  
Principal Scientist and Group Leader  
Ossianix

Larry Brown  
Executive Vice President R&D, Chief Scientific Officer  
Noveome Biotherapeutics

Martin Tolar  
Founder, President & CEO  
Alzheon, Inc.

Massimiliano Bianchi  
General Manager & Scientific Director  
Transpharmation Ltd

Ottavio Vitolo  
VP Clinical Development  
Homology Medicines Inc.

Patrice Garnier  
CEO  
Amabiotics

Ping Chiao  
Senior Director  
Biogen

Richard Ransohoff  
Vice President & Senior Research Fellow – Neuroimmunology  
Biogen

Richard Wade-Martins  
Professor Molecular Neuroscience  
University of Oxford

Robert Bell  
Senior Principal Scientist & Lab Head of Neurovascular Biology  
Pfizer

S. Pablo Sardi  
R&D Director  
Sanofi-Genzyme

Samuel Hasson  
Principal Investigator, Neuroscience  
Pfizer

Susan Browne  
Director  
Teva Pharmaceuticals

Tamara Maes  
CSO and VP  
Oryzon Genomics S.A

Tarek Samad  
Head, Neurodegeneration  
Pfizer

Tricia Thornton Wells  
Senior Investigator, Neuroscience Disease Area Portfolio Leader for Biomarker Development  
Novartis IBR

Essentially every speaker was excellent. A very informative and thought provoking series of presentations.

National Institute Neurologic Disease & Stroke
Pre-Conference Workshop Day | Tuesday, February 20

Workshop A
Exploring the Application of Gene Editing in the Treatment of CNS Disorders
8.00am - 11.00am

Gene editing holds the potential for a transformative approach to the therapy of CNS disorders. The emergence of novel gene-editing tools such as CRISPR, TALEN, Zinc Finger Nucleases and Homologous Recombination opens new possibilities in the development of disease relevant animal models and, more importantly, in highly specific therapeutic interventions.

In order for these technologies to come to fruition, several challenges need to be addressed, including efficient delivery to target areas and selective editing of neuronal and non-neuronal cell populations.

This workshop will introduce the potential applications of the gene-editing technology in rare neurodegenerative diseases from preclinical study design to therapeutic potential.

Join the session to:
- Uncover protocol advancements for advanced in vitro/vivo editing with optimized specificity
- Learn about the different applications of CRISPR, TALEN and AAV-edited transgenic mice in a biomedical research setting
- Overcome the current limitations of using gene editing tools for insertions, for higher efficiency of desired edits

Workshop B
The Promise & Challenge of Neuroimmune Therapeutics: Targets, Models, and the Science Behind the Innate Immune System’s Role in Neurodegenerative Disease
11.30am - 2.30pm

Whilst microglia have long been implicated in the pathology of neurodegenerative disease, recent accumulating evidence has pointed to these cell types as a driving factor in neuronal damage and disease progression for AD, PD and others.

Although different models have been established to study these diseases and cell type strategies, their effectiveness has been limited. In order to understand the interplay of underlying mechanisms and to investigate new therapeutic modalities, there is a need to increase human-relevance and accuracy. Recent technological advances, including iPSCs, now grant access to substantial quantities of disease-pertinent neurons, both with and without predisposing mutations.

Join this session to:
- Understand the accumulation of genetics and human biology implicating innate immune function as being integral to diseases such as Alzheimer’s and Parkinson’s
- Discuss mechanisms and the biological pathways linked to the human genetics of these diseases
- Therapeutic targets and levers – where could we intervene to alter microglial phenotypes and modify disease progression?

Workshop Leader
Ottavio Vitolo, VP
Clinical Development,
Homology Medicines

Dr. Vitolo is a neuropsychiatrist and researcher with extensive pre-clinical and clinical research experience in both academic and industry settings. He is currently Assistant Psychiatrist at Massachusetts General Hospital, and Vice President for Clinical Development at Homology Medicines, a private biotechnology company focused on gene therapy and gene editing for rare diseases. Previously he has held positions of increasing responsibility at Pfizer, including Senior Medical Director and Head of Neuromuscular Clinical Research, as well as Head of Implementation for Rare Neuromuscular Disease programs targeting treatments for diseases including Duchenne Muscular Dystrophy, Huntington’s disease and schizophrenia. He was also formerly Associate Medical Director of Discovery Research at Shire Human Genetic Therapies.

Workshop Leader
Samuel A. Hasson
Principle Scientist, Lab Head
Pfizer Internal Medicine

Samuel Hasson is a Lab Head within Pfizer Neuroscience in Cambridge, MA focused on developing human genetics-informed targets in Alzheimer’s and Parkinson’s disease into therapeutic programs. In particular, he is involved in the understanding of how microglia in the CNS can play a protective role against disease pathogenesis and progression. Before joining Pfizer, Samuel’s previous work encompassed the molecular aspects of mitochondrial quality control and the translational potential of mitophagy in Parkinson’s disease.
Emerging data suggests that there is a link between changes in the gut microbiome and neurological diseases and disorders. This exciting bi-directional communication via the proposed gut-brain axis or GBA, has catapulted microbiota into the scientific spotlight for CNS disorders. However, with the size and complexity of the Gut-Brain Axis, there still remains a number of fundamental questions to allow scientific researchers to forge a successful path in demonstrating causality for a number of disease indications and ultimately developing target specific therapeutics.

As these two hot areas of research come together, now is the time to understand how we can continue to fuel this dynamic relationship.

Join this session to:

• Receive an overview of recent scientific advances in unravelling the gut-brain connections and potential pathways to harness these findings for novel therapeutic approaches
• Consider different approaches to modulation or supplementation of the microbiome
• Discuss the challenges associated in developing microbiota-associated therapeutics and strategies to overcome them
• Hear perspectives of microbiota-associated therapeutics in the personalized medicine era

The Gut-Brain Axis: The Microbiome as a Versatile Target for Neurodegenerative Disease

3.00pm - 6.00pm

Workshop Leader

Patrice Garnier
CEO
Amabiotics

Dr. Patrice Garnier is Chief Executive Officer for Amabiotics (www.amabiotics.com), a biopharmaceutical company that develops innovative diagnostics and microbiome-derived medicines to fight age-related diseases with a strong research focuses on gut-brain axis pathologies, metabolic disorders and oncology. The leading drug candidate, AMA-101, is a potential first-in-class therapy for Parkinson’s disease.

Dr. Garnier is an entrepreneur with 20 years of experience leading high-tech companies. Prior to joining Amabiotics in 2013, he founded and served for 9 years as CEO in a bioinformatics company that delivers genome to metabolome solutions for data analysis and management. He also co-founded IgenBio (www.igenbio.com) in 1993, a US based company which develops genome analysis products and services for the life science industry.

Dr. Garnier holds an MSc in quantum physics from the École Normale Supérieure in Paris. He completed his PhD in nanotechnology in Professor Catherine Brechignac’s group at the Laboratoire Aimée Cotton, CNRS.
### Conference Day One | Wednesday, February 21

#### 8.25 Chairs Welcoming & Opening Remarks

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#### Novel Therapeutic Approaches for Targeting Neuroinflammation & Neurodegeneration

<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speakers</th>
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<tbody>
<tr>
<td>8.25</td>
<td>Chairs Welcoming &amp; Opening Remarks</td>
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<tr>
<td>8.30</td>
<td>(Re) Introduction to Microglia as Intrinsic Brain Cells</td>
<td>Richard Ransohoff – Vice President &amp; Senior Research Fellow – Neuroimmunology Biogen</td>
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<tr>
<td></td>
<td>• Demonstrating the role of microglia enter the developing CNS in early embryogenesis and carry out crucial developmental tasks</td>
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<td></td>
<td>• Highlighting how basal and reactive microglial transcriptomes point towards mechanisms underlying functions</td>
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<td>• Showcasing how our microglial research tool kit continues to grow but still lacks some desirable utensils</td>
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<td>8.50</td>
<td>Targeting Innate and Adaptive Immune Mechanisms to Modulate Neurodegeneration</td>
<td>Tarek Samad – Head, Neurodegeneration Pfizer</td>
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<td></td>
<td>• Reviewing accumulating evidence suggesting that proliferation of microglia has an important role in neurodegeneration</td>
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<td></td>
<td>• Demonstrating data showing innate and adaptive immune mechanisms as important factors in controlling proliferation and function of microglia in AD</td>
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<td>9.10</td>
<td>Shedding Light on the Role of TREM2 in Alzheimer’s Disease</td>
<td>Damian Crowther – Director R&amp;D Neuroscience IMED Biotech Unit AstraZeneca</td>
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<td>• Demonstrating how TREM2 shedding from the microglia leaves them “blind” to debris in the brain</td>
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<td>• Exploring how shed TREM2 is increased in neurological diseases and accelerated shedding is linked to Alzheimer’s disease</td>
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<td>• Proteolysis occurs at a single position in the protein, providing a target for therapy</td>
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<td>9.30</td>
<td>The Importance of Microtubular Proteins &amp; Cytokines in Neuroplasticity &amp; Neuroinflammation</td>
<td>Massimiliano Bianchi – General Manager &amp; Scientific Director Transpharmation Ltd</td>
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<td></td>
<td>• Exploring the interplay between alterations in microtubular proteins and cytokines in neuroinflammation and neuroplasticity</td>
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<td>• Demonstrating CSF data highlighting the relationship between a-syn, Tau and microtubules and their effects on plasticity</td>
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<td></td>
<td>• Evaluating the interaction between microtubular proteins and cytokines and possible pharmacological interventions</td>
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<tr>
<td>9.50</td>
<td>Session Q&amp;A Panel</td>
<td>Richard Ransohoff – Biogen</td>
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<td></td>
<td>• What is the pathological role of neuroinflammation and the immune system in the development of neurodegenerative diseases?</td>
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<td></td>
<td>• How should we be targeting and/or developing dynamic therapeutic strategies for tackling neuroinflammation?</td>
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<td></td>
<td>• How can genetics drive the identification of novel targets for immune-based therapy towards microglial and neuroinflammation in neurodegenerative disorders?</td>
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<tr>
<td>10.30</td>
<td>Speed Networking &amp; Morning Refreshments</td>
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### Discovery Track

**Novel Approaches to Target & Phenotypic Based Discovery**

<table>
<thead>
<tr>
<th>Time</th>
<th>Title</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>11.30</td>
<td>Systems Biology Approaches to Accelerating Neurodegenerative Drug Discovery</td>
<td>Alice Zhang, CEO, Verge Genomics</td>
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<td></td>
<td>Demonstrating how large-scale genomic datasets can be used to tackle three core challenges in CNS drug discovery and development:</td>
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<td>- Identifying next-generation therapeutic targets; Selection of appropriate preclinical disease models; Patient enrichment strategies and biomarker discovery</td>
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<td>- Exploring how can patient derived iPSC's can be combined with patient genetic and gene expression data to create an “all-in-human” approach to drug discovery</td>
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<td><strong>11.50 Transcriptomic Profiling &amp; Experimental Validation to Explore Drug Repurposing in Parkinson’s Disease</strong></td>
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<td></td>
<td>Exploring the value of using iPSC-derived dopamine neurons generated from Parkinson’s patients in research</td>
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<td>Highlighting the importance of experimental validation of an in silico prediction in robust models of Parkinson’s</td>
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<td></td>
<td>- The central role of lysosomal biology in Parkinson’s</td>
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<td></td>
<td>- Does transcriptomic analysis of disease models provide a short-cut to drug repurposing?</td>
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<td><strong>12.10 Modulation of Histone Modifying Enzymes for Treatment of Neurodegenerative Disease</strong></td>
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<td>Demonstrating the modulation of the histone lysine demethylase, LSD1, has positive effects on cognition and neuroinflammation in different animal models of neurodegenerative disease</td>
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<td>ORY-2001 has finalized Phase I studies and is ready for the trials in patients with neurodegenerative disease</td>
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<td><strong>12.30 Session Q&amp;A Panel</strong></td>
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<td>Where can genetics and big data be best utilized to drive the identification of novel pathway and target selections?</td>
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<td>- Can genetic profiles be utilized to identify higher risk patient populations in earlier phases of disease initiation?</td>
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<td>- How should we factor in the dynamic relationships, epigenetic drivers and pathology of neurodegenerative disease?</td>
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<td>- How does the heterogeneity of AD and other degenerative diseases dictate focusing on distinct subpopulations?</td>
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<td>Alice Zhang, Verge Genomics, Richard Wade-Martins, University of Oxford, Tamara Maes, Oryzon Genomics S.A.</td>
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### Development Track

**Therapeutic Strategies for Effectively Crossing the Blood Brain Barrier**

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<th>Time</th>
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<th>Speaker(s)</th>
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<tr>
<td>11.30</td>
<td>Single Domain Antibody Platform for Delivery of Biologics to the CNS</td>
<td>Krzysztof Wicher, Principal Scientist and Group Leader, Ossianix</td>
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<td>Combination of in vivo and in vitro phage selections allowed for identification of efficient, cross-species reactive, and safe CNS shuttles specific to TFR1 receptor</td>
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<td>The shuttle mediates uptake of small peptides, antibodies and enzymes to the brain parenchyma, where these cargos can exert their physiologic/therapeutic action</td>
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<td>11.50</td>
<td>Strategies to Improve Penetration &amp; Delivery to the Brain</td>
<td>Robert Bell, Senior Principal Scientist &amp; Lab Head of Neurovascular Biology, Pfizer</td>
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<td>Highlighting current data on innovative technological approaches such as chemical modifications and physical disruption for the delivery of therapeutics to the CNS</td>
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<td>- Exploring novel shuttle and trojan approaches for delivery of therapeutics across the brain-blood barrier</td>
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<td><strong>12.10 Nose to Brain Delivery of Neuroprotective Amnion Derived ST266 Secretome</strong></td>
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<td>Intranasal delivery is an effective means to deposit large molecular weight proteins to the brain</td>
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<td>Amnion derived secretome is neuroprotective and anti-inflammatory in optic nerve disease and traumatic injury models</td>
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<td><strong>12.30 Session Q&amp;A Panel</strong></td>
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<td>What passive and active mechanisms should be considered during the development and delivery of effective therapeutic interventions to central nervous system targets?</td>
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<td>- How can we best harness innovative methods including chemical modifications, Trojan horse approaches, physical targeting and disruption, nanoparticles, ultrasound, and other technologies?</td>
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<td>- What potential opportunities are there to catalyze development of novel treatments that cross the BBB from the preclinical to clinical phase with an emphasis on risks, levers, and potential collaborative efforts among sectors</td>
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<td>Krzysztof Wicher, Ossianix, Robert Bell, Pfizer, Larry Brown, Noveome Biotherapeutics</td>
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**World CNS**

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1.00 Lunch & Networking

Addressing The Need for Integrative Disease Modelling

2.00 Progress and Challenges in the Development of Translational in Vivo Disease Modification Assays
- Reviewing approaches to demonstrate disease modification in preclinical in vivo assays for neurodegenerative disorders
- Exploring progress and hurdles towards translational assays: keeping the clinical goal in sight
- Optimizing the application of currently available in vivo assays to exploit their strengths
- Highlighting Huntington’s disease, ALS, and Parkinson’s disease research

Susan Browne
Director
Teva Pharmaceuticals

2.20 Profiling Specific Cell Populations in Human Brain Tissue to Identify New Targets
- Demonstrating new insights about healthy and disease states as well as aging through Cerevance’s molecular profiling of neuronal and glial cell nuclei
- Highlighting how our large-scale approach provides an alternative to relying on animal models, iPS cells and single cell analysis
- Through comparative analysis, we are identifying potentially new drug targets that are highly selectively expressed in cell populations and circuitry that are most vulnerable in neurodegenerative diseases

Brad Margus
Co-founder & CEO
Cerevance

2.40 Session Q&A Panel
- *In vitro, in vivo and/or in silico* analyses: where should we be applying our resources to bridge the translational gap in neurodegenerative research?
- How can the study of genotype-phenotype interactions be integrated to enhance the translation of modelling systems?
- Are we able to cost-effectively scale up advanced blood-brain barrier models to make high throughput and or high content screening a reality?
- Which of these dynamic models most effectively and accurately models the blood-brain barrier to prove selective drug delivery and distribution?
- What advances have been made using human iPSc-derived neurons to model key mechanisms of pathology and their applications to stimulate drug discovery?

Susan Browne
Director
Teva Pharmaceuticals

Brad Margus
Co-founder & CEO
Cerevance

3.00 Afternoon Refreshments & Networking

Exploring the Utility of Non Human Primate Models in the Study of Neurodegeneration

4.00 Evaluating Translatability: Relevance of Rodent & Nonhuman Primate Preclinical Models of Cognitive Impairment to Clinical Drug Development
- Reiterating cognitive impairment as a key component of various CNS disorders as well as an important potential adverse effect of medications
- Reviewing similarities and differences in homology between rodent/human and nonhuman primate/human in relationships between brain organization and cognitive capacity
- Highlighting advantages and disadvantages of rodent and nonhuman primate models of cognitive dysfunction
- Demonstrating translational successes/failures of rodent and nonhuman primate cognition models

Jay Schneider
Professor, Pathology, Anatomy and Cell Biology, Director, Parkinson’s Disease Research Unit
Thomas Jefferson University
4.20 Translational Value of Non-Human Primate Models of Motor Impairment and Disease Progression in Parkinson’s Disease

• Demonstrating the value of MPTP-lesioned non-human primates in predicting Phase II efficacy of novel treatments for motor symptoms, and motor side-effects of treatment, in Parkinson’s disease
• Reviewing the limitations of MPTP-lesioned non-human primate in predicting Phase II efficacy in providing disease modifying benefit in Parkinson’s disease
• Exploring recent progress and applications of alpha-synuclein-based non-human primate models for evaluating novel approaches to disease modification in Parkinson’s disease

4.40 Session Q&A Panel

• How are new technologies converging to enhance primate specific neurodegenerative research?
• Despite advances, how should we seek to overcome the technical challenges in the creation and analysis of transgenic primate models?
• For which applications should primate models act as a critical tool in the advancement of neurodegenerative research?
• How do we build the business case for their use, given cost and ethical considerations amongst recent advances in the development of lower order and cellular based models?

5.00 Closing Remarks & Close of Conference Day One
Conference Day Two | Thursday, February 22

8.25 Chairs Opening Remarks

Developing Next Generation Directed Therapies for Aβ Tau in Alzheimer’s

8.30 The Amyloid Cascade Hypothesis: The Path Forward in Developing Targeted Therapies for Alzheimer’s Disease
- Highlighting strategic benefits of applying genomic stratification of patients for validation of biomarkers from target discovery to therapeutic development
- Describing discovery of a novel molecular mechanism of action of ALZ-801, which blocks formation of toxic amyloid oligomers associated with development and progression of Alzheimer’s disease
- Reviewing development of ALZ-801, a Phase 3-ready, first-in-class, small molecule oral inhibitor of amyloid aggregation and neurotoxicity
- Emphasizing need for application of precision medicine approach in neurodegeneration, based on individual genetic and biological information, to advance therapies with the greatest impact for patients

Martin Tolar
Founder, President & CEO
Alzheon, Inc.

Jan Torleif Pedersen
Director, Disease Biology Lead, Alzheimer’s Disease and Dementia, H. Lundbeck A/S

8.50 Development of Phospho-Tau Antibodies Targeting Pathologic Forms of Tau
- Demonstrating that microtubule binding protein Tau is hyperphosphorylated and aggregated during neurodegenerative processes such as Alzheimer’s disease
- Revealing data showing hyperphosphorylated aggregated forms of Tau as being able to act as an endopathogen in preclinical models of tauopathies
- Showcasing the development of highly specific and selective monoclonal antibodies which will target hyperphosphorylated aggregated forms of Tau. These antibodies will prevent seeding of pathology in preclinical models of tauopathies

Martin Tolar
Alzheon, Inc.

Jan Torleif Pedersen
H. Lundbeck A/S

9.10 Session Q&A Panel
- Does Amyloid Beta really still remain the holy grail of treatment targets?
- Is there a need to apply the combinatorial therapy paradigm to neurodegenerative diseases? If so, how?
- What preclinical and clinical funding strategies are available to maximize resources for drug discovery and development?

9.30 Morning Refreshments & Networking

Discovery, Development & Validation of Non-Invasive Signatures as Prognostic & Diagnostic Biomarkers of Disease

10.30 Perspectives on Biomarker Applications Through Drug Development
- Exploring the utility of biomarkers and imaging for early development and in late clinical development
- Divulging where we are with ‘tool box use’ of these markers for various diseases and the current obstacles and limiting factors to be overcome for regulatory adoption

Johan Luthman
Vice President, Neuroscience Business Group
Eisai Pharmaceuticals

10.50 Amyloid PET Imaging in Aducanumab Ph1b PRIME study
- Showcasing recent data from the PRIME study, highlighting aducanumab as an Aβ-removing and potentially disease-modifying therapy for AD
- Presenting supportive evidence, showcasing how amyloid PET has been effectively utilized as an informative clinical biomarker

Ping Chiao
Senior Director, Biogen

11.10 Target Engagement: The Missing Link in Drug Development Programs
- Demonstrating the role of target engagement biomarkers in drug development
- Understanding how target engagement is defined and should be demonstrated

Jeffrey Cummings
Director
Cleveland Clinic

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### 11.30 Session Q&A Panel

- What remain the analytical and regulatory considerations for incorporating neuroimaging systems to advance biomarker monitoring?
- How can we drive innovation with imaging systems for more accurate disease identification and characterisation?
- Can we effectively combine fMRI and PET imaging to develop better indicators of cognitive function for neurodegenerative diseases?
- How should we approach issues around validation and quantification of diagnostic biomarkers?

### 12.00 Lunch & Networking

### Exploring Advances in Emerging CSF-Based Biomarkers

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<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
<th>Affiliation(s)</th>
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<tbody>
<tr>
<td>1.00</td>
<td>Utility of Neurofilament Light Chain as a Translational Tool in Neurodegenerative Disorders</td>
<td>Holly Soares</td>
<td>Head, Translational Neuroscience</td>
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<td>Head, Translational Neuroscience</td>
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<td></td>
<td>Douglas Galasko</td>
<td>Professor, Department of Neurosciences, University of California San Diego</td>
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<td>1.20</td>
<td>Biomarker Data in Cerebrospinal Fluid in Parkinson’s Disease: The PPMI Study</td>
<td>Tricia Thornton Wells</td>
<td>Senior Investigator, Neuroscience Disease Area Portfolio</td>
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<td>Leader for Biomarker Development</td>
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<tr>
<td>1.40</td>
<td>Using Genetics for Patient Selection and Outcome Prediction in Complex, Heterogeneous Neurological Disorders</td>
<td>Holly Soares</td>
<td>AbbVie</td>
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<td>Douglas Galasko</td>
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<tr>
<td>2.00</td>
<td>Session Q&amp;A Panel</td>
<td>Holly Soares</td>
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### 2.30 Afternoon Refreshments & Networking
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<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>3.00</td>
<td>Developing Preclinical Biomarkers of Proteopathic Seeds in Neurodegenerative Disease</td>
<td>Jonathan Levenson, Senior Director, Preclinical Research &amp; Development, Proclara Biosciences</td>
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<tr>
<td></td>
<td>• Reviewing disease association with misfolding and aggregation of a number of different proteins</td>
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<td>• Highlighting the pathological role of spread, at least in part, due to transmission of proteopathic seeds from cell-to-cell</td>
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<td>• Exploring how next-generation fluid biomarkers for neurodegenerative diseases should track the transmissible form pathological proteins</td>
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<td>3.20</td>
<td>The Role of GBA in Lysosomal Function, Neuroprotection and Progression in Parkinson’s Disease</td>
<td>S. Pablo Sardi, R&amp;D Director, Sanofi-Genzyme</td>
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<td>• Highlighting clinical, genetic and experimental evidence underlies the relevance of lysosomal dysfunction in Parkinson’s disease</td>
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<td>• Showcasing how stimulation of the lysosomal GBA pathway in the CNS can improve the pathological and behavioral abnormalities in preclinical models of disease</td>
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<td>• Revealing the potential impact of modulating this lysosomal pathway to slow the progression of Parkinson’s disease is being studied in a genetically defined population</td>
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<td>3.40</td>
<td>AAV2-AADC Gene Therapy for Advanced Parkinson’s Disease: Interim Data, Clinical Development Insights</td>
<td>Brendon Boot, Medical Director, Voyager Therapeutics</td>
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<td>• Demonstrating the potential for gene therapy to restore lost CNS function by creating an alternative source of the enzyme responsible for the conversion of levodopa to dopamine (AADC)</td>
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<td>• Exploring updated biomarker and clinical interim data readouts from VY-AADC01 clinical studies</td>
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<td>• Discussing unique aspects of clinical development required in “one-and-done” therapies</td>
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<td>4.00</td>
<td>Targeting Autophagic Protein Clearance Mechanisms for the Treatment of Neurodegenerative Disease</td>
<td>Douglas Bonhaus, CSO, Neuropore Therapeutics</td>
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<td>• Demonstrating the rationale for developing targeted therapeutics for autophagy</td>
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<td>• Strategies for tackling the related challenges both preclinically and clinically</td>
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<td>• Presenting the story of the discovery and validation of data from therapeutics directed at LAMP2a, PI3K and/or TLR 2</td>
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<td>4.20</td>
<td>Session Q&amp;A Panel</td>
<td>Jonathon Levenson, Proclara Biosciences, S. Pablo Sardi, Sanofi-Genzyme, Brendon Boot, Voyager Therapeutics, Douglas Bonhaus, Neuropore Therapeutics</td>
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<td>• Although spread offers a unifying pathophysiological concept of neurodegenerative diseases, are the mechanisms of spread universal?</td>
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<td>• How can advanced research showing the propagation of spread in Parkinson’s be applied to AD and other neurodegenerative diseases?</td>
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<td>• What importance does the biochemical characteristics of the proteopathic seeds hold? E.g. are oligomers, are more detrimental to cells than are fibrillar forms?</td>
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<td>• To what relevance and extent does lysosomal dysfunction, glial disruptions etc have on the transmission of protein pathology in neurodegenerative diseases?</td>
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<tr>
<td>5.00</td>
<td>Chairs Closing Remarks</td>
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<td>5.05</td>
<td>Close of Conference</td>
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PARTNER WITH US

PARTNERS

Expertise Partner: Atuka
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 cutting-edge, 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.
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Spun-out of GlaxoSmithKline Pharmaceuticals in 2010, Transpharmation Ltd is a CRO with a difference; crosstherapeutic area expertise in CNS translational pharmacology from bench to bedside. With cutting-edge laboratories and capabilities in England and Ireland, Transpharmation has made a significant impact in CNS Contract Research. With decades of Drug Discovery expertise; science-focused relationships and best-in-class assays are its hallmark; serving international clients across AD, PD, Cognitive Disorders, Schizophrenia, Treatment Resistant Depression, Sleep Disorders, pharmacoEEG, Schizophrenia, Epilepsy, Pain and Pharmacodynamic modelling.
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DELEGATE SPLIT BY INDUSTRY:

Pharma 45%
Biotech 20%
Academic & NFP Institutions 24%
Solution Providers 11%

DELEGATE SPLIT BY SENIORITY:

C-Level 26%
VP 10%
Director 40%
Scientist 24%

The World CNS Summit 2018 is a unique meeting of pharma, biotech and academia, designed to help scientists and business leaders in these organizations overcome the barriers to translational research within neurodegenerative diseases.

Delegates are actively seeking outside expertise, collaborators and partners to help them:

• Utilize translatable, in-silico, in-vitro and in-vivo models of neurodegenerative disease for more predictive and efficient safety and efficacy testing
• Identify novel biomarker strategies for accurately interpreting target engagement, pharmacodynamics, disease progression and disease diagnostics
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* Audience breakdown from 2017 meeting
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**VENUE**

_The Westin Copley Place_
10 Huntington Avenue, Boston, MA, 02116, United States

For further information or assistance, please visit www.westincopleyplaceboston.com

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