February 20-22, 2017, Boston, MA

World CNS Summit 2017

Targeting Neurodegenerative Diseases

The 5th Annual World CNS Summit: Dedicated to Transforming Translational & Clinical Research

Hear from 36+ World Class Scientists Including:

Frank Bennet
Senior Vice President
Ionis Pharmaceutical

Ole Isacson
Senior Vice President & Chief Scientific Officer, Neuroscience & Pain Research Unit
Pfizer

Sophie Parmentier-Batteur
Director, Early Discovery
Merck

Jang-Ho Cha
Global Head Translational Medicine, Neuroscience
Novartis Institute for BioMedical Research

Johan Luthman
VP Neuroscience Clinical Development
Eisai Pharmaceuticals

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The absolute best neuroscience meeting I have attended in years

Rene Anand, Professor,
Department of Pharmacology,
Ohio State University

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Book early and save up to $500
Welcome to World CNS 2017

Challenge traditional thinking around the development of effective treatments for neurodegeneration

Neurodegenerative diseases are evidently on the rise. Alzheimer’s Disease alone is predicted to increase by 100% in America over the next 10 years.

Yet, despite heavy investment, there remains a lack of effective disease-modifying therapeutics for neurodegeneration.

Is this a failing of our understanding of the pathological mechanisms of disease? Or is the trial process effectively flawed?

The World CNS Summit brings together scientific thought leaders from academia, pharma and biotech actively working in the neurodegenerative space to address these critical questions. Revealing details of the latest breakthroughs and discoveries, this meeting will challenge traditional thinking and approaches in the development of neurodegenerative therapeutics.

Join your peers to examine novel therapeutic targets and innovative therapeutic approaches, as well as advances in biomarker development, neuroimaging and clinical trial redesign.

The interactive nature of the meeting will enable you to collaborate to tackle critical pre-clinical and clinical barriers you are facing, with the aim of streamlining translational research across the field.

Hear What Previous Attendees Have To Say:

- Top notch presentations and discussions
  - AbbVie

- An excellent meeting from a scientific and clinical perspective
  - Cure Parkinson’s Trust

- The meeting provided a great opportunity to network and provided exposure to aspects of neuro research beyond my own sphere of expertise
  - Genzyme

Top 10 Reasons to Attend the World CNS Summit 2017:

1. Harness innovation in novel therapeutic strategies targeting neurodegeneration to progress disease modifying therapies.

2. Address the dichotomy between symptomatic and preventative therapies across neurodegenerative diseases.

3. Utilize the achievements and lessons learnt in the development of therapeutics for orphan-orphan and orphan diseases.

4. Tackle the challenge of crossing the blood brain barrier and achieving more on-target therapeutic delivery.

5. Discover how to utilize a multitude of modeling techniques to increase translatability and seamlessly transition from pre-clinical to clinical drug development.

6. Advance translational biomarker discovery and development from target engagement to disease progression.

7. Understand how the precision medicine paradigm is revolutionizing R&D in the neurodegenerative field.

8. Employ advances in neuroimaging technology to drive biomarker and drug development.

9. Redefine the use of pre-clinical models of neurodegeneration to enhance their application in translational research.

10. Design truly patient-centric trials with data collection and monitoring devices that prioritize patient convenience.

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Speakers

Ole Isacson  
Senior Vice President & Chief Scientific Officer, Neuroscience & Pain Research Unit  
Pfizer

Spyros Papapetropoulos  
VP & Global Head, Neurodegenerative Diseases, Movement Disorder & Clinical Research Transformation  
Teva Pharmaceuticals

John Geisler  
Chief Scientific Officer  
Mitochon Pharmaceuticals, Inc.

Frank Bennet  
Senior Vice President Research, Neurology Franchise Leader  
Ionis Pharmaceuticals

Jang-Ho Cha  
Global Head Translational Medicine, Neuroscience  
Novartis Institute for BioMedical Research

Lee Henderson  
CEO  
Vybion, Inc.

Johan Luthman  
VP Neuroscience Clinical Development  
Eisai Pharmaceuticals

Susan Browne  
Director Early Discovery  
Teva Pharmaceuticals

Pablo Sardi  
R&D Director, Neuroscience  
Sanofi-Genzyme

Richard Wade-Martins  
Professor of Molecular Neuroscience  
Oxford Parkinson’s Disease Centre

Andrea Edling  
Associate Scientific Director  
Sanofi

Richard Mohs  
Chief Scientific Officer  
GAP Foundation

Gregory Stewart  
Consultant  
Alchemy Neuroscience

Daniel Michaelson  
Professor of Neurobiology  
Tel Aviv University

Yoshi Bando  
Associate Professor  
Asahikawa Medical University

Kuldip Dave  
Director Research Programs  
Michaeal J. Fox Foundation

Steven Braithwaite  
CSO  
Alkahest, Inc

Georgia Mitsi  
Sr. Director, Search & Evaluation, Digital Healthcare Initiatives  
Sunovion Pharmaceuticals Inc

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<table>
<thead>
<tr>
<th>Name</th>
<th>Title and Organization</th>
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<tbody>
<tr>
<td>Michael C. Irizarry</td>
<td>VP, Early Clinical Development, Neurosciences, Eli Lilly and Company Ltd</td>
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<tr>
<td>Massimiliano (Max) Bianchi</td>
<td>General Manager &amp; Scientific Director, Transpharmation, University of Florida College of Medicine</td>
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<tr>
<td>Michael Hutton</td>
<td>CSO, Neurodegenerative Diseases DHT, Eli Lilly &amp; Company Ltd</td>
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<tr>
<td>Vaibhav Diwadkar</td>
<td>Associate Professor, Wayne State University School of Medicine</td>
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<td>Sam Agus</td>
<td>Chief Specialist, Medical Affairs, Neurology, H. Lundbeck A/S</td>
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<td>Nikolai Naryshkin</td>
<td>Senior Director, Biology – Genetic Disorders, PTC Therapeutics, Inc</td>
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<td>Doug Bonhaus</td>
<td>Interim CEO, Neuropore</td>
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<tr>
<td>Jonathan Levenson</td>
<td>Senior Director, Proclara Biosciences</td>
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<td>Matthew Kennedy</td>
<td>Director, Neuroscience, Merck Research Laboratories</td>
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<tr>
<td>Dorothy Schafer</td>
<td>Assistant Professor of Neurobiology, University of Massachusetts Medical School</td>
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<tr>
<td>Toby Ferguson</td>
<td>Medical Director, Neurology, Biogen</td>
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<tr>
<td>Hien Zhao</td>
<td>Senior Scientist, Ionis Pharmaceuticals</td>
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<td>Edward Wild</td>
<td>MRC Clinician Scientist, UCL Institute of Neurology</td>
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<td>Brendon Boot</td>
<td>Medical Director, Voyager Therapeutics, Inc.</td>
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<tr>
<td>Tricia Thornton-Wells</td>
<td>Investigator III, Neuroscience Disease Area, Portfolio Leader, Novartis Institutes for Biomedical Research</td>
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<td>Stephen Arnericic</td>
<td>Executive Director, Coalition Against Major Diseases (CAMD), Critical Path Institute</td>
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<td>Claudio Babiloni</td>
<td>Professor, University of Rome “La Sapienza”</td>
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Conference Day One | Tuesday, February 21, 2017

8.30 Chairman’s Opening Remarks

Improving Translatability from Drug Design to the Clinic

8.40 Animal Models of Parkinson’s Disease: State of the Field & the Future!
- Evaluating standardized phenotypic characterization of rodent models of Parkinson’s disease
- Exploring independent replication of pre-clinical models using contract research laboratories
- Adding value and utility of pre-clinical models for researchers seeking to test potential therapeutics targeting alpha-synuclein

Kuldip Dave, Director Research Programs, Michael J. Fox Foundation

9.10 Utilizing Patient Derived iPS cells as a Means to Enhance Translatability
- Understanding how to best apply iPSC modeling to advance drug discovery and development efforts
- What advances have been made in the development of relevant assays using iPSC-derived human neurons
- Exploring the impact of iPSC derived neuron models in supporting the discovery of disease specific therapeutic targets

Richard Wade-Martins, Professor of Molecular Neuroscience, Oxford Parkinson’s Disease Centre

9.40 Panel Discussion: Where Are We Now - Developing Appropriate Translational Models for Multifactorial Pathology of Neurodegenerative Disease
- Reviewing current pre-clinical models and determining what questions we want these models to answer
- Assessing how we can begin to get more disease relevancy into the pipeline earlier- is the future in in-silico, in-vitro or in-vivo models
- Understanding the limits of our current models and rationalizing whether current mice models are sufficient for translational research
- Questioning the growing chorus to shift away from animal models and exploring whether in-vitro models really replace in-vivo models
- Identifying triggers of disease to better inform disease modeling
- Analyzing the strengths and weaknesses of different pre-clinical modeling systems to advance pre-clinical translation

10.25 Morning Refreshments & Speed Networking

The meeting content was great

Upsher Smith Laboratories
## Novel Therapeutic Targets for the Treatment of Neurodegenerative Disease

### AD / PD

**11.25 Targeting Lysosomal Defects in the Treatment of Parkinson’s Disease: From Genetics to Therapeutics**
- Exploring clinical, genetic and experimental evidence that underlies the relevance of lysosomal dysfunction in Parkinson’s disease
- Highlighting the importance of the study of rare diseases as a new paradigm for drug discovery
- Assessing how displaying the stimulation of the lysosomal GBA pathway in the CNS can improve the pathological and behavioral abnormalities in animal models of disease
- Understanding how modulation of this lysosomal pathway may represent a new disease-modifying treatment for GBA-related Parkinson’s disease

**Pablo Sardi**, R&D Director, **Sanofi-Genzyme**

**11.25 Huntington’s Disease as a Proving Ground for Novel Target & Delivery Strategies**
- Spearheading the development of novel biomarkers, targeted therapeutics and delivery methods
- Developing next-generation molecular genetic therapeutics and the methodology to deliver and test them efficiently
- Paving the way for accelerated development of such treatments across neurodegeneration

**Edward Wild**, MRC Clinician Scientist, **UCL Institute of Neurology**

**11.55 Inhibitors of Leucine-rich Repeat Kinase 2 (LRRK2): Progress & Promise for the Treatment of Parkinson’s Disease**
- Reviewing the role of LRRK2 in the pathogenesis of Parkinson’s Disease
- Exploring the progress for antisense as therapeutics for CNS disorders
- Illustrating the progress and promise of antisense therapeutics directed at LRRK2 in models of Parkinson’s Disease

**Hien Zhao**, Senior Scientist, **Ionis Pharmaceuticals**

**11.55 Strategies for the Development of ALS Therapeutics for Novel Targets**
- Addressing current challenges in ALS development
- Discussing pre-clinical strategies to improve target selection
- Evaluating clinical strategies to improve ALS trials

**Toby Ferguson**, Medical Director, Neurology, **Biogen**

**12.25 Looking Towards APOE4 for the Treatment of Alzheimer’s Disease**
- Examining the need to focus on distinct subpopulations with Alzheimer’s such as APOE4 due to the heterogeneity of the disease
- Assessing how the myriad of suggested APOE4 driven mechanisms is converging towards potential therapies
- Exploring mechanism based anti APOE4 directed therapies in development

**Daniel Michaelson**, Professor of Neurobiology, **Tel Aviv University**

**12.25 The Emergence of Gene Therapy for CNS Disease & Its Use for Delivering a New Drug Class: A Novel Target that Reveals New Details of Huntington’s Disease Pathogenesis**
- Evaluating current gene therapy approaches for CNS disease
- Discussing the use of Intrabodies, intracellular antibody fragments, and their potential advantages in preventing CNS disease progression
- Demonstrating the potential for INT41 as a therapeutic agent in Huntington’s disease by targeting a toxic degradation fragment in the mutant huntingtin protein which interferes with the transport to the nucleus

**Lee Henderson**, Chief Executive Officer, **Vybion, Inc.**

**12.55 Lunch & Networking**
# Universal Strategies for Approaching Targeting Neurodegeneration

## Bioenergetics & Mitochondrial Dysfunction

### 1.55 The Disease Modifying Effects of a Mitochondrial Specific Protonophore, MP101, a Broad Spectrum Treatment for Insidious Neuromuscular, Neurodegenerative & Developmental Diseases by Modulating Mitochondrial Physiology

- MP101 modulates the entire organelle physiology, improving mitochondrial dysfunction, neuronal survival and behavior in models of Huntington Disease, Rett Syndrome and Duchenne Muscular Dystrophy
- MOA stems from the induction of increased energy expenditure, BDNF expression, lowering ROS production, and remodeling, initiating as a non-genomic
- Since the actions are initially non-genomic vs. a protein pathway and this organelle is highly evolutionarily conserved, there are considerable advantages that predict strong translation, precisely our next step!

- **John Geisler**, Chief Scientific Officer, Mitochon Pharmaceuticals, Inc.

## Neuro-inflammation & Microglial Activation

### 1.55 Can Neuroinflammation Imbalances Provide the Triggers & Mechanisms for Synaptic and Neuronal Loss in Parkinson’s and Alzheimer’s disease?

- Assessing how brain protein and lipid abnormalities can create significant inflammatory pre-conditions to neurodegeneration
- Exploring how signals of inflammatory responses in brain glia and cytokines can be modified by new agents to reduce neurodegeneration and help clearance of pathological stimuli
- Looking to peripheral and central biomarkers related to neuroinflammation, lipid and protein abnormalities to help define diagnostic indices and provide the most precision and impact to human conditions

- **Ole Isacson**, Senior Vice President & Chief Scientific Officer, Neuroscience & Pain Research Unit, Pfizer

## Targeting Mitochondrial Dysfunction to Rectify Abnormal Morphology of Myelin & Axon Pathology Associated with MS

- An insight into the role morphological abnormalities of myelin and axonal organelles play in the pathogenesis of axonal injury in demyelinating diseases
- Describing the central role of mitochondria in axonal degeneration and on pathogenesis of MS

- **Yoshi Bando**, Associate Professor, Asahikawa Medical University

## Modulating Microglia-Neural Circuit Interactions: Novel Therapeutic Approaches for Targeting Neurological Disorders

- Exploring new roles for microglia in modulating development and plasticity of neural circuits
- Identifying novel molecular mechanisms by which microglia remodel synaptic connectivity
- Translating basic mechanisms by which microglia regulate neural circuit plasticity to neurodegenerative disease

- **Dorothy Schafer**, Assistant Professor of Neurobiology, University of Massachusetts Medical School

## Targeting Mitochondrial Dysfunction in Neurodegenerative Disease

- Exploring the contributions of mitochondrial dysfunction to disease progression in muscular dystrophy
- Examining a variety of therapeutic approaches to the problem
- Highlighting recent small molecule data in a severe mouse model of muscular dystrophy

- **H. Lee Sweeney**, Professor & Director of the UF Myology Institute, University of Florida College of Medicine

## Targeting Innate Immune Cells as a Novel Therapeutic Approach for Neuro-inflammation & Neurodegeneration

- Understanding the pathological role of neuro-inflammation and the immune system in the development of neurodegenerative diseases
- Evaluating the impact of innate immune mechanisms and microglia activation in neurodegeneration
- Identifying CSF-1R as a potential target for therapeutic modulation of CNS innate immune cells for Multiple sclerosis and neurodegenerative diseases

- **Andrea Edling**, Associate Scientific Director, Sanofi

## 3.25 Afternoon Refreshments & Poster Session
**Paving the Way to Preventative Medicine: Novel Therapeutic Approaches Towards Neurodegeneration**

### Gene, Anti-sense & Other Emerging Therapies

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<tr>
<td><strong>4.00</strong></td>
<td><strong>AAV2-AADC Gene Therapy for Parkinson’s Disease: Therapeutic Potential &amp; Surgical Coverage of the Putamen using MRI-Guided Convective Delivery</strong></td>
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<td><strong>4.30</strong></td>
<td><strong>The Potential for Antisense Therapies in Addressing Severe Neurological Disorders such as SMA</strong></td>
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<tr>
<td><strong>5.00</strong></td>
<td><strong>Plasma Derived Therapeutics for Neurodegeneration</strong></td>
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### Small Molecule Approaches

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<td><strong>Investigating the Efficacy &amp; Safety of Verubecestat in Alzheimer’s Disease Patients</strong></td>
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<td><strong>4.30</strong></td>
<td><strong>Development of a Small Molecule Alpha-Synuclein Stabilizer for the Treatment of Parkinson’s Disease</strong></td>
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<tr>
<td><strong>5.00</strong></td>
<td><strong>Targeting Pre-mRNA Splicing With Small Molecules for the Treatment of Neuromuscular Disorders</strong></td>
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**4.00** AAV2-AADC Gene Therapy for Parkinson’s Disease: Therapeutic Potential & Surgical Coverage of the Putamen using MRI-Guided Convective Delivery

- Exploring the potential for gene therapy with adeno-associated virus 2 (AAV2) carrying the gene for AADC in enhancing dopamine production and improving the response to levodopa in advanced PD
- Displaying the impact of real-time MRI guidance in enabling accurate cannula placement and real-time adjustment to maximize anatomical coverage and delivery of vector to the putamen
- Identifying advances in surgical techniques that have markedly improved vector delivery and increased coverage of the putamen

**Matthew Kennedy**, Director, Neuroscience, *Merck Laboratories*

**4.30** The Potential for Antisense Therapies in Addressing Severe Neurological Disorders such as SMA

- Understanding the potential of antisense for CNS disorders
- Updates from the ENDEAR phase 3 clinical program, and looking towards nusinersen in the broader SMA population

**Frank Bennet**, Senior, Vice President Research, Neurology Franchise Leader, *Ionis Pharmaceuticals*

**5.00** Plasma Derived Therapeutics for Neurodegeneration

- Understanding how the analysis of the plasma proteome in aging has provided two major opportunities to derive therapeutics: plasma fractions and antidotes against aging factors
- Gaining an insight into Alkahest’s controlled clinical studies with young plasma
- Exploring their validated strategies involving plasma fractions, as well as small molecule antagonists against aging factors

**Karoly Nikolich**, CEO, *Alkahest, Inc*

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**4.00** Investigating the Efficacy & Safety of Verubecestat in Alzheimer’s Disease Patients

- Exploring the discovery of verubecestat and its clinical status for AD
- Looking forward to the next generation of BACE inhibitors and identifying strategies for their application

**4.30** Development of a Small Molecule Alpha-Synuclein Stabilizer for the Treatment of Parkinson’s Disease

- Oligomeric forms of alpha synuclein are toxic and likely contribute to the pathology and progression of Parkinson’s disease (PD)
- Exploring several rational approaches to treating PD: prevent formation, enhance clearance, reduce toxic consequences of these aggregates
- Presenting the story of the discovery and validation of a molecule that prevents the formation of a particularly toxic form of alpha synuclein aggregate

**Doug Bonhaus**, Interim CEO, *Neuropore*

**5.00** Targeting Pre-mRNA Splicing With Small Molecules for the Treatment of Neuromuscular Disorders

- The potential of small molecules for therapeutic targeting of pre-mRNA splicing
- Targeting alternative splicing of SMN2 exon 7 for treatment of spinal muscular atrophy
- Targeting alternative splicing of exon 20 in mutant IKBKAP gene for treatment of familial dysautonomia

**Nikolai Naryshkin**, Senior Director, Biology – Genetic Disorders, *PTC Therapeutics, Inc*

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**5.30** Chairman’s Closing Remarks

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**5.35** End of Conference Day One
**Conference Day Two | Wednesday, February 22, 2017**

**Chairman's Opening Remarks**

**Strategies Targeting Aβ Tau**

**From Biomarkers to Diagnostics: Applications from Target Engagement to Patient Stratification**

8.40

- Johan Luthman, VP Neuroscience Clinical Development, Eisai Pharmaceuticals

- Addressing the need for the co-development of biomarkers and drug development
- Exploring perspectives on companion diagnostics in neurodegeneration

**Targeting the Spread of Pathology in Neurodegenerative Diseases**

9.10

- Jonathan Levenson, Senior Director, Proclara Biosciences

- Elucidating biological triggers in the spread of neurodegeneration
- Highlighting screens for candidate therapeutics must be based around the biological mechanisms involved in spread of pathology
- Exploring in vitro and cell-based screens that model spread of Aβ, tau and synuclein pathology as well as the translation of in vitro screens into animal models
- Identifying novel strategies for targeting the spread of pathology to block the progression of neurodegeneration

**Microtubular Proteins & Cytokines as Peripheral Biomarkers of Disease Progression in Neurodegenerative Disorders: A Novel Path Towards Innovative Pharmacological Interventions?**

9.40

- Massimiliano (Max) Bianchi, General Manager and Scientific Director, Transpharmation Ltd

- Explore alterations in microtubular proteins and cytokines in neurodegenerative disorders
- Analyze Tubulin post-translational modifications and TAU in plasma and CSF: from preclinical models to clinical settings
- Evaluate interaction between microtubular proteins and cytokines and possible pharmacological interventions

**Identification of Novel Therapeutic Targets Against Tau Pathology in Disease-Relevant Cellular Models**

10.10

- Sophie Parmentier-Batteur, Director, Neuroscience Discovery, Merck

- Exploring examples of human iPSC-derived neurons to model key mechanisms of tau pathology and their applications to stimulate drug discovery
- Utilizing phenotypic chemical or genetic screens to identify novel targets against tau pathology that have better chance of clinical translatability
- Understanding the interplay between tau, amyloid and APOE4 to advance the translational development of therapeutic interventions

**The Challenges & Potential of Combination Therapies for Alzheimer’s Disease**

11.20

- Michael C. Irizarry, VP, Early Clinical Development, Neurosciences, Eli Lilly and Company
- Michael Hutton, CSO Neurodegenerative Diseases DHT, Eli Lilly and Company Ltd

- Understand the rationale for exploring combination therapies targeting different mechanisms in the AD pathogenic process
- Evaluate an example of transgenic mouse studies that demonstrate synergistic clearance of existing amyloid plaques by combining treatments targeting different parts of the amyloid cascade
- Highlight the challenges in developing a robust AD model that recapitulates both amyloid and tau pathologies (and their interaction) to assess combinations targeting both amyloid and tau preclinically
- Review development and regulatory approaches for combination therapies in Alzheimer’s Disease
### 11.50 Breakout Roundtable Discussions

This session will focus on devising solutions that cannot be obtained in presentation format, and will give you actionable takeaways to build a more holistic understanding of the field. Access the critical information that is only obtainable through frank and open discussion around crucial topics in the field. Shape the debate around the following topics:

1. **The Need for Integrative Disease Modeling & Precision Medicine**
   - Ole Isacson, Senior Vice President & Chief Scientific Officer, Neuroscience & Pain Research Unit, Pfizer

2. **Addressing the Dichotomy Between Disease Modification & Symptomatic Treatments**
   - Brendan Boot, Medical Director, Voyager Therapeutics, Inc.

3. **Achieving Targeted Drug Delivery to the Brain**
   - Gregory Stewart, Consultant, Alchemy Neuroscience

4. **Pre-clinical & Clinical Funding Strategies to Maximize Resources for Drug Discovery & Development**
   - Sam Agus, Chief Specialist, Medical Affairs, Neurology, H. Lundbeck A/S

5. **What is the Future for Neurodegenerative Therapeutics?**
   - Sophie Parmentier-Batteu, Director, Neuroscience Discovery, Merck

6. **Is Aβ Tau all it’s Cracked up to be?**

### 12.30 Lunch & Networking

#### Driving Success in the Clinical Landscape

<table>
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<tr>
<th>Time</th>
<th>Topic</th>
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<tbody>
<tr>
<td>1.30</td>
<td><strong>Computational Analysis &amp; Quantitative Models of Biomarkers to Inform Efficacy &amp; Patient Stratification in Clinical Trials</strong></td>
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<td>• Outline the breadth of biomarkers in use and under development across the AD field</td>
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<td>• Examine issues related to using biomarkers for patient stratification</td>
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<td>• Explore the consequences of not having the right biomarkers</td>
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<td>• Examine the virtues and perils of data mining and restricted hypothesis testing</td>
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<td><strong>From Neuro-architectures to Biomarkers: Network from Neuroimaging Dysfunction from Neuroimaging Data &amp; its Relevance for Understanding Neurodegenerative Syndromes</strong></td>
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<td>• Exploring a framework to search for more meaningful biomarkers that more accurately capture the mechanisms of the complex organ that is the human brain</td>
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<td>• Gaining an insight into network dynamics at macro-sopic [temporal and spatial] scales that are driven by behavioral task demands</td>
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<td></td>
<td>• Presenting data from multiple cognitive/behavioural domains (memory, motor and attention) and in healthy and pathological populations</td>
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2.30 Panel Discussion: Incorporating New Diagnostic Capabilities to Advance Biomarker Monitoring
• Exploring the known neurophysiological and metabolic bases of currently employed in vivo functional imaging techniques
• How can we maximize the use of neuro and molecular imaging for risk reduction and identification of cognitive symptom onset early on

3.00 Afternoon Refreshments & Networking

3.30 The Need for Novel Endpoints in Neuroscience Clinical Trials
• Addressing current success rates in neuroscience drug development which are too low to be sustainable
• Neuroscience clinical trials rely on clinical rating scales which are limited by high variability and low signal detection
• Evaluating modern technologies that hold promise for more direct, more continuous assessment of disease severity and medication effect
• Exploring how integrating novel endpoints could revolutionize drug development in Neuroscience

4.00 Re-wiring Clinical Development in CNS: Biometric Monitoring, Smart & Virtual trials
• Questioning how biometric tech platforms can contribute to clinical development and can ultimately replace the search for accurate surrogate biomarkers?
• Exploring how we can make clinical trials ‘smarter’ as well as identifying what components are needed for trial virtualization
• Addressing whether or not patients are ready

4.30 Harnessing Big Data & Wearable Technology to Advise Clinical Readouts
• Debating big data vs. objective measures for specific conditions
• Evaluating wearable devices consumer vs. medical grade; what should we use and when?
• Understanding the difference between what patients are willing to use and what researchers are looking for

5.00 Chairman’s Closing Remarks

5.05 End of Day Two & Close of Conference
Dementia due to neurodegenerative diseases has been the graveyard for many promising compounds.

A recent study reported that out of the 244 compounds (413 clinical trials) for the care of Alzheimer’s disease between 2002 and 2012, only one was approved. Alzheimer’s disease drug candidates have 99.6% of the failures rate, more than any disease area (81% for cancer).

Furthermore, Dementia is one of the main medical burdens for the global economy with £350 billion invested each year. Pharma companies need incentives to invest in research with the risk not to find a cure or disease-modifying therapy in the coming years.

The creation of a transcontinental Public-Private entity is a promising solution to speed up research. This will enable harmonized and cross-validated research that can improve the identification of novel biomarkers and effective drug development pathways.

However, this raises many questions such as:

- Is it feasible and realistic?
- How can we create a world class alliance that is able to orchestrate the sharing of pre-competitive pre-clinical and clinical data among industry, regulatory authorities, and academia?
- What would be a reasonable roadmap for this purpose?

The session will review the experience of four successful Public-Private entities created with such a mission at a European and USA level - namely the Intelligent Medicine Initiative (IMI), The Coalition Against Major Diseases (CAMD) of C-Path, and the Accelerating Medicines Partnership - Alzheimer’s Disease (AMP-AD) and the Global Alzheimer’s Platform Foundation (GAP-NET).

Engage with fellow peers to discuss topics and ways to align the distinct continental Public-Private entities. Share lessons learned, and brainstorm possible solutions towards the creation of a transcontinental Public-Private Entity in this field.

### Pre-Conference Workshop Day

**A Trans-Continental Pre-Competitive Public-Private Entity Leveraging Open Science to Develop Effective Biomarkers For Use In Drug Development: Dream or Possible Reality?**

**Date:** February 20, 2017 | **Time:** 9:00am-3:40pm

Claudio Babiloni, Professor, University of Rome “La Sapienza”

**9.00** Chairman’s Opening Remarks

Sam Agus, Chief Specialist, Medical Affairs, Neurology, H. Lundbeck A/S

**9.10** Alzheimer’s Disease – Not Only a Development Challenge!

- What happens when a disease modifying treatment (DMT) is approved?
- How many patients will get the treatment?
- The biggest gap is, understanding who is the patient

**9.40** Roundtable Discussions

Addressing the Clinical Trial Landscape

Developing Novel Non Invasive Surrogate Biomarkers

**10.40** Morning Refreshments & Networking

Claudio Babiloni, Professor, University of Rome “La Sapienza”

**11.30** Intelligent Medicine Initiative (IMI) as a Public-Private Space for Drug Discovery in Europe
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<td>The Accelerating Medicines Partnership-Alzheimer’s Disease (AMP-AD) as a Public-Private Space for Pre-clinical Validation in Drug Discovery in USA</td>
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<tr>
<td>12.10</td>
<td>Foundational Regulatory Science Providing Actionable Solutions for Unmet Needs in Alzheimer Disease</td>
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<td>• Examples highlighting the impact that could not have been achieved by one individual organization alone</td>
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<td>• Exploring CAMD development of global consensus clinical data standards for Alzheimer’s disease (CDISC) including:</td>
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<td>• Development of unified clinical trial database consisting of placebo data from multinational clinical trials</td>
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<td>• Regulatory qualification and endorsement for neuroimaging biomarkers for enrichment in early AD clinical trials</td>
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<tr>
<td>12.30</td>
<td>Global Alzheimer’s Platform Foundation (GAP-NET) as a Public-Private Space for Coordinating Stakeholders Initiatives Against Alzheimer’s Disease in USA</td>
</tr>
<tr>
<td>1.00</td>
<td>Lunch &amp; Networking</td>
</tr>
<tr>
<td>2.00</td>
<td>Workshop Discussion with Audience:</td>
</tr>
<tr>
<td>3.30</td>
<td>Chairman’s Closing Remarks</td>
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<tr>
<td>3.40</td>
<td>Close of Pre-Conference Workshop Day</td>
</tr>
</tbody>
</table>

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
## Partners

### Hosting Partner

**Roche Diagnostics**  
Along with Roche Pharmaceuticals, Roche Diagnostics is an important part of the foundation that modern healthcare builds upon. Our broad range of innovative diagnostics tests and systems play a pivotal role in the ground-breaking area of integrated healthcare solutions and cover the early detection, targeted screening, evaluation and monitoring of disease. Roche Diagnostics is active in all market segments, from scientific research and clinical laboratory systems to patient self-monitoring.  

www.roche.com

### Industry Partner

**Pfizer Inc.**  
About Pfizer Inc.: Working together for a healthier world® At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world’s best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world’s premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us.  

www.pfizer.com

### Expertise Partner

**Transpharmation**  
Spun-out of GlaxoSmithKline Pharmaceuticals in 2010, Transpharmation Ltd is a CRO with a difference; cross-therapeutic 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.  

www.transpharmation.co.uk

### Why Partner

The World CNS Summit 2017 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.
- Streamline and **optimize clinical trial design**, managing the rigorous approval standards demanded in neurodegenerative trials.
- Incorporate the use of **digital and mHealth technology** for use in ‘smart trials’ to reduce costs, enhance data collection or boost enrolment.
- **Identify novel biomarker strategies** for accurately interpreting target engagement, pharmacodynamics, disease progression and disease diagnostics.

### Contact us today to discuss how you can get the best from this opportunity.

- A great meeting. The networking opportunities were fantastic.  
  **Pfizer**

- One of the best CNS therapeutics meetings to attend.  
  **P2D Bioscience**  

### Become a Partner

**Contact**

Mo Langhi  
Commercial Director  
Tel: +1 212 537 5898  
Email: sponsor@hansonwade.com

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Email: register@hansonwade.com
Mail: Hanson Wade
4th Floor, 52 Grosvenor Gardens,
London, SW1W 0AU

<table>
<thead>
<tr>
<th>Team Discounts*</th>
<th>Top 3 Benefits of Attending</th>
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<tbody>
<tr>
<td>2 Delegates – 25% off Second Delegate</td>
<td>1. Harness innovation in novel therapeutic strategies targeting neurodegeneration to progress disease modifying therapies</td>
</tr>
<tr>
<td>3 Delegates – 25% off Second Delegate + 50% off Third</td>
<td>2. Utilize the achievements and lessons learnt in the development of therapeutics for orphan-orphan and orphan diseases</td>
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<thead>
<tr>
<th>Industry</th>
<th>Register &amp; Pay before Friday January 13, 2016</th>
<th>Standard Prices</th>
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<tr>
<td>Gold Package Conference + Workshop Day</td>
<td>$3298 (save $300)</td>
<td>$3398 (save $200)</td>
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<tr>
<td>Silver Package Conference Only</td>
<td>$2499 (save $200)</td>
<td>$2699</td>
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<tr>
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<td>$2899 (save $100)</td>
<td>$2999</td>
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<tr>
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<td>$999</td>
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*Academics are entitled to a 40% discount off the Industry Pricing using the code ‘ACA’ (Please note: No further discounts are applicable)

Venue

The Westin Copley Place
10 Huntington Avenue
Boston, MA, 02116
United States

www.westincopleyplaceboston.com

Please note: Overnight accommodation and travel are not included in the registration fee.

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 15 days before the conference attendees will receive a full credit to a future conference. Cancellations received 15 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.

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

National Institute Neurologic Disease & Stroke