

World CNS Summit 2017

Targeting Neurodegenerative Diseases



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Breakthroughs in several bona fide pathological mechanisms that cause and/or contribute to neurodegeneration.

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

Evidence of disease modification in a neurodegenerative disorder

Susan Browne, Director Early Discovery, **Teva Pharmaceuticals**

How we interrogate disease modifying therapies in presymptomatic subjects at risk as well as seeing the development of combination strategies to study amyloid and tau directed therapies

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

A better understanding of the roles immune cells play in disease onset with a goal of developing better biomarkers and detection of early stage disease.

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

The breakthrough I would like to see would be for scientists to step outside of their silos and consider other approaches to finding cures. The sheer amount of clinical failures and loss of money spent on Alzheimers with redundant approaches trying to mop up plaques and tangles suggests the issue is somewhere else. I thought that the paper by Dr. Virginia Lee showing that isoprostanes, a biomarker of reactive oxygen species, are significantly elevated in a model of AD and precede plaque formation by 4-months, would have been a red herring that the issue is upstream at the mitochondria.

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

We need to understand selective vulnerability: why do some neurons die and some do not, and what role does ageing play? In Parkinson's we know some dopamine neurons die and some are protected. Exploiting the molecular mechanisms underlying that protection would make an exciting route towards a neuroprotective therapy.

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

In NDDs, the "one size fits all" model has significantly reduced our chances of success. In the next 3-5 years, we'll start seeing results from the first genetic-based trials. The promise of precision medicine for NDDs will be at close reach.

S. Pablo Sardi, R&D Director **Sanofi Genzyme**

I would like to see the advancement of disease modifying treatments for neurodegenerative disorders to serve the large unmet need in patient populations.

Ole Isacson, SVP & CSO, Neuroscience & Pain Research Unit, **Pfizer**

Demonstration of the clinical benefit of a new disease modifying therapeutic strategy in one of the major degenerative disease (AD, PD, ALS).

Sophie Parmentier Batteur, Director, Early Discovery, **Merck**

Better ways to measure disease severity and progression that do not rely on investigator-administered rating scales.

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

Gaining a better understanding of the pathological mechanism(s) leading to the selective degeneration of neurons in ALS, HD, AD, and PD.

Gregory Stewart, Consultant, **Alchemy Neuroscience**

I think one of the big open questions in the field that I hope will become elucidated in the next five years is a better understanding of adult onset neurological disorders such as Huntington's and Alzheimer's diseases and ALS, especially for known autosomal dominant mutations – why does it take decades for the disease to manifest? What are the roles of developmental factors and modifier genes? How would this new knowledge affect the diagnosis, management and clinical trial design in each of such diseases?

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

To join the conversation, submit your answer to the question here info@hansonwade.com