Collaboration, Innovation and Commitment

Welcome to our first volume of Research ALS Today, marking an exciting time for ALS research. The goal of this new publication is to challenge and inform scientists to become ALS investigators. With the availability of model systems both in vivo and in vitro mimicking aspects of the disease, and advances in technology, new partnerships among academia, industry, government and not-for-profit groups allow experts from diverse fields to collaborate toward new treatments.

ALS (Amyotrophic Lateral Sclerosis), also known as motor neuron disease or Lou Gehrig’s disease, is a complex fatal disorder affecting motor neurons and neighboring glia in the brain and spinal cord, leading to cell death and muscle paralysis. This complexity invites innovation and collaboration.

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Christopher Henderson, Columbia University

The ALS Association joins the American Academy of Neurology in presenting The 2007 Sheila Essey Award for ALS Research to Christopher E. Henderson, Ph.D., during the Academy’s 59th Annual Meeting in Boston, April 30 through May 4.

Henderson, formerly at INSERM (France’s Institut National de la Santé et de la Recherche Médicale), and now at Columbia University in New York City, studies the way that nerve cells die in a process called programmed cell death, and how that process interacts with the inflammation that accompanies the cell loss in neurodegenerative diseases. Many of his investigations have been funded by The ALS Association and have yielded important insight into why the disorder kills motor neurons.

“I see our work as one example of how studying the basic mechanisms of development and cell death can lead to findings that are relevant to late-onset diseases such as ALS,” Henderson said. “The role of patient-related organizations such as The ALS Association, has been critical: supporting the basic work needed for discovery, while encouraging and facilitating the translation toward more direct applications.”

The cell death process is triggered when a death receptor is activated by nitric oxide.

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Henderson cont.

Henderson and colleagues detected an amplified nitric oxide response in mice expressing mutant SOD1 (copper-zinc superoxide dismutase 1—a mutation linked to some forms of ALS) well before symptoms could be detected in these mice.

In keeping with a growing appreciation of the role of glial cells in the demise of motor neurons in ALS, neighboring astrocytes and microglia appear to secrete nitric oxide as well, Henderson found.

“As co-founder of Trophos, Henderson is an excellent example of a scientist who sees the bigger picture,” noted Lucie Bruijn, Ph.D. “His expertise helped drive the development of motor neuron assays for drug discovery, which has led to a lead compound currently in clinical trials for ALS.”

The $25,000 prize honors the memory of Sheila Essey and was made possible through the generosity of the Essey Family Fund. Past recipients have often used the funds to support research of promising young scientists on their teams.

In a focused drug discovery effort funded by TREAT ALS, proprietary stem cells and gene manipulation technologies will be combined in a search for targets for candidate therapies for the disease.

Partnering with The ALS Association are the companies Galapagos NV and Stem Cell Innovations, Inc. The three million dollar milestone driven project will be directed by a team of experts with a goal to find new targets for the disease in the next two years.

The unique ability provided by SCI to screen human motor neurons on a large scale, and Galapagos' target discovery technology will open new approaches to developing therapies for ALS. This alliance is an important initiative within The ALS Association’s mission to find a cure for and improve living with ALS.

Stem Cell Innovations has a proprietary stem cell technology based on cells that are exempt from the President’s ban. The stem cells are able to produce human motor neurons that can grow robustly in the lab.

“My colleague Dr. James Kelly and I are very proud to work with The ALS Association in the fight against ALS,” said Onno van de Stolpe, CEO of Galapagos. “This alliance builds on both our CNS expertise and on our strong franchise in working with non-profit health organizations to identify disease-modifying drug targets for unmet medical needs.”

TO KEEP CURRENT with the ALS field, read the monthly journal news reports at www.alsa.org under the research tab.

RESOURCES Visit www.alsa.org and click on the blue research tab for these resources:

- SOD1 mutations database www.alsod.org
- Coriell NINDS DNA repository http://ccr.coriell.org/ninds/
- ALS Epidemiology http://aces.stanford.edu/ForRes.html
- SOD1 mutant rats, Taconic, http://www.taconic.com/wmspage.cfm?parm1=258
- SOD1 mutant mice, The Jackson Laboratory http://jaxmice.jax.org/models/als.html