

Project A.L.S. Opens Privately-Funded Stem Cell Research Lab

The Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research Opens in New York — The First to Focus Exclusively on ALS and Related Diseases

MAY 15, 2006 — Project A.L.S. today opens the first privately-funded lab to focus exclusively on the study of stem cells to treat ALS (amyotrophic lateral sclerosis) and related motor neuron diseases, announced Valerie Estess, Research Director for Project A.L.S.

The Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research (Project A.L.S. Lab) is a joint venture between Project A.L.S. and Columbia University. Based in New York, the Project A.L.S. Lab does not accept federal funding and has an “open-door” policy that encourages Project A.L.S.-funded stem cell researchers and collaborators from Harvard University, Johns Hopkins University, the Salk Institute, Memorial Sloan-Kettering Cancer Center, and other New York-based institutions to collaborate with Columbia University-based scientists and clinicians.

“The creation of a state-of-the-art laboratory where the latest ideas in stem cell biology can be explored on human stem cell derived motor neurons, without hindrance or constraint, is a very remarkable achievement,” said Thomas M. Jessell, Ph.D., a Howard Hughes Medical Institute Investigator, Professor of Biochemistry and Molecular Biophysics and a Member of the Center for Neurobiology and Behavior at Columbia University Medical Center in New York City.

“The entire ALS community, within New York City and nationally, owes Project A.L.S. a big debt of gratitude. Their far-sighted and pro-active research agenda is setting the standards for modern approaches to investigating neurodegenerative diseases.”

Christopher E. Henderson, Ph.D., and Hynek Wichterle, Ph.D., of Columbia University will serve as senior scientific advisors to the Project A.L.S. Lab. Dr. Jessell will be the research advisor. These appointments recognize the long-standing relationship between Columbia University’s world-renowned neuroscientists and Project A.L.S.

In 1999, Project A.L.S. recruited Dr. Jessell’s laboratory to examine how stem cells could help scientists better understand and treat ALS. Since that time, Drs. Jessell and Wichterle’s research has demonstrated that embryonic stem cells can be directed to differentiate into functional motor neurons, the very nerve cells selectively destroyed in ALS. The Project A.L.S. Lab provides a dedicated place for exploration of these findings and for work toward treatments and cures for people with ALS and related motor neuron diseases.

Dr. Jessell continued, “In establishing the lab, Project A.L.S. has paved the way for a dramatic acceleration in the pace at which advances in basic motor neuron biology can be translated into more effective clinical therapies for the treatment of amyotrophic lateral sclerosis and related diseases.”

Initially, the research initiatives of the Project A.L.S. Lab will include:

- The development of the first human cell-based ALS assays or tests, which will provide new information about the human form of the disease. (Previously, scientists have studied transgenic mice that develop ALS.);
- The use of stem-cell-derived motor neurons to screen drug candidates for ALS and SMA (spinal muscular atrophy);

Subsequently, the work of the Project A.L.S. Lab will move toward development of stem cell transplantation studies.

The Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research is named in memory of Project A.L.S. co-founder, Jenifer Estess, who was diagnosed with ALS at the age of thirty-five, and who died from the disease in 2003. ALS is a uniformly fatal neurodegenerative disease related to Alzheimer's and Parkinson's, and results in progressive paralysis of nearly all voluntary muscles, including ones controlling movement, speech, and breathing. Also known as Lou Gehrig's disease, ALS will affect more than 300,000 Americans living now.

"The Project A.L.S. lab will be a crucial resource...it is extraordinary that Project A.L.S. has implemented this so rapidly. This bodes well for accelerated development of stem cell therapeutics in ALS," said Robert H. Brown, Jr, M.D., D.Phil., Professor of Neurology at Harvard Medical School, and a Project A.L.S. collaborator.

Project A.L.S. has a record of supporting promising research in its earliest stages. In 1998, Project A.L.S. was first to unite and fund a working group of stem cell biologists, ALS clinicians, anatomists, and basic scientists to better understand whether stem cells could be a valuable tool to unlock the mysteries of ALS and related diseases.

In only seven years, the Project A.L.S. stem cell consortium has achieved significant scientific breakthroughs, including:

- In 2002, Hynek Wichterle (Columbia University) et al. showed that stem cells could be directed to become bone fide motor neurons, the very cells that are destroyed in ALS and related motor neuron diseases (Cell, 2002, Aug 9)
- In 2003, a Project A.L.S.-funded team led by Doug Kerr and researchers from Johns Hopkins University showed that rats paralyzed with an ALS-like syndrome regained significant motor function after receiving an infusion of stem cells into the spinal fluid (Journal Neuroscience, 2003 June 15).
- Collaborator Robert M. Brownstone et al. showed that embryonic-stem-cell-derived motor neurons could establish appropriate connections with target muscle (Journal Neuroscience, 2004 Sep 8; Journal Neuroscience, 2006 Mar 22).
- Collaborators including Drs. Fred Gage (Salk Institute), Christopher Kintner (Salk Institute) and Sally Temple (Albany Medical College) are characterizing endogenous populations of stem cells and looking ahead to using these cells as tools toward regeneration and repair.

"Project A.L.S. is thrilled to open the first stem cell lab of its kind, not for the bells and whistles that come along with a grand opening, but because Project A.L.S. has shown that stem cells will very likely make a difference in understanding and treating a fatal human disease," Valerie Estess said. "The Project A.L.S. Lab is our latest—and most significant—investment in stem cell research. It is the necessary next step in forwarding scientific discovery, and, one day, stopping brain disease."