

# Project A.L.S.

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## **Jenifer Estess Laboratory for Stem Cell Research Announces First Disease-Specific Stem Cells from Human Skin Cells**

**Research Team Creates Human ALS Motor Neurons, Providing New  
Opportunities for Disease Modeling, Drug Testing, and  
Personalized Regenerative Medicine.**

NEW YORK - A team of researchers from the Harvard Stem Cell Institute (HSCI) and Columbia University, in a collaboration catalyzed by the Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research, has demonstrated that pluripotent stem cells generated from a patient with ALS (amyotrophic lateral sclerosis) can be directed to differentiate into motor neurons—the very brain cells destroyed by ALS. The results of the team’s study appear in today’s online issue of *Science*. This is the first published report to show that disease-specific stem cells may be derived from an individual patient.

In the study, led by Kevin Eggan, of the Harvard Stem Cell Institute, skin cells taken from a patient with a familial form of ALS were induced to become pluripotent stem cells. Scientists then differentiated the pluripotent cells into motor neurons and glia (support cells in the brain) that featured an ALS genotype.

“This is a seminal discovery,” said Valerie Estess, director of research for Project A.L.S. “The ability to derive ALS motor neurons through a simple skin biopsy opens the doors to improved drug discovery. For the first time, researchers will be able to look at ALS cells under a microscope and see why they die. If we can figure out how a person’s motor neurons die, we will figure out how to save motor neurons.”

Starting in 1999, Project A.L.S. recruited leading scientists and clinicians to define the potential role of stem cells in understanding and treating ALS, the fatal neurodegenerative disease, also known as Lou Gehrig’s disease. Project A.L.S.-funded scientists began by transplanting stem cells directly into mice with ALS, with limited success. More recent

experiments have shown that stem cells may be more valuable as tools to understand the disease process and create mini-representations of disease—or assays—for the purpose of drug screening.

“For the first time, we have the opportunity to examine cellular and molecular defects in motor neurons and glial cells derived from patients with ALS. And we can now begin drug screens on disease-specific classes of human motor neurons,” said Thomas Jessell, a Howard Hughes Investigator at Columbia University, and Project A.L.S. advisor. “Through the work of the Jenifer Estess Laboratory for Stem Cell Research we now can glimpse the new age of ALS research, an age of progress and promise.”

Co-author on the paper, Christopher Henderson, who is co-director of the Columbia University Center for Motor Neuron Biology and Disease, and senior scientific advisor to the Project A.L.S. Laboratory, said: “It has been a privilege to collaborate with Kevin Eggan and his team and to contribute to this critical step forward. We will continue to work hand-in-hand with Harvard researchers and Project A.L.S. to exploit the potential of these cells for drug screening”.

Three years ago, Project A.L.S. asked Dr. Eggan, a stem cell expert, and Chris Henderson, Hynek Wichterle, as authorities on motor neuron biology and drug screening at Columbia University, to work together to understand ALS, one of our most complicated and devastating neurological disorders. Today’s publication marks the first major breakthrough of this collaboration.

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Project A.L.S. is a non-profit 501©3 whose mission is to understand, treat, and cure ALS, also known as Lou Gehrig’s disease. The hallmark of the company’s approach is collaboration between researchers and clinicians, many of whom have not focused on ALS specifically, or worked together before. In ten years, Project A.L.S. has raised over \$37 million for research worldwide. Located in New York, the Project A.L.S./Jenifer Estess Laboratory for Stem Cell is the world’s only privately funded laboratory to focus exclusively on stem cell and ALS. The laboratory was named for Project A.L.S. founder Jenifer Estess, who died from ALS in 2003.

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