

# Stem cells provide new tool for studying disease and identifying ALS drugs

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Results of two studies funded by Project A.L.S. and appearing in today's advance online publication of *Nature Neuroscience* demonstrate that embryonic stem cells may provide a new tool for studying disease mechanisms and for identifying drugs to slow ALS, also known as Lou Gehrig's disease.

Both studies were completed by researchers participating in an ongoing collaboration with the Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research, the world's first and only privately funded laboratory focused exclusively on stem cells and ALS.

The complementary studies, led by Kevin Eggan, of the Harvard Stem Cell Institute, and Serge Przedborski, of Columbia University Medical Center compellingly demonstrate that embryonic stem cells can be used to create an in vitro model of ALS, a fatal neurodegenerative disease that selectively destroys motor neurons, messenger cells responsible for virtually all voluntary movement.

Until now, scientists have not known whether motor neurons in ALS die because of a problem within the cell—or from outside the cell. The study by Eggan's group describes successful use of a novel embryonic stem cell-based model for ALS that will help scientists to answer this and other questions. Utilizing this model both Eggan and Przedborski's groups observed that non-neuronal cells called astrocytes may have a toxic effect on motor neurons in ALS.

The Columbia study provides further evidence that astrocytes are toxic to motor neurons in ALS. The Columbia team's discovery of astrocyte-toxic mediators provides not only an insight into how the damage associated with ALS occurs, and a potential biomarker for early diagnosis of the disease — but also provides a target for potential new therapies aimed at slowing motor neuron degeneration in ALS.

"That's what happens when scientists from major institutions work together toward shared goals. Project A.L.S. prides itself on building productive research teams from elegant parts," said Valerie Estess, director of research for Project A.L.S.

"The remarkable interest, curiosity and open-mindedness for new and promising lines of research is emblematic of Project A.L.S.," added Serge Przedborski, Page and William Black Professor of Neurology, at Columbia.

Source: PROJECT A.L.S.

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