

Project A.L.S.

Two Project A.L.S. Studies Confirm Reliability of Human iPS Cells, Opening Door for ALS Modeling and Drug Screening

February 4, 2011 –In 2008, *Time* magazine named “First Neurons Created from ALS Patients” its #1 scientific and medical breakthrough of the year. While many were hopeful that iPS cells represented a giant step toward personalized medicine that would allow scientists to model human ALS and other diseases, iPS cell lines generated in several laboratories have exhibited instability and behavioral variability.

In two compelling studies published online today, a team of scientists from the Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research, the Broad Institute, Harvard Stem Cell Institute, the Motor Neuron Center at Columbia University, and the Howard Hughes Medical Institute, among other institutions, announced that it has established reliable procedures for evaluating the efficiency of differentiated *induced pluripotent stem* (iPS) cell lines and *embryonic stem* (ES) cell lines, and have begun to use the cells lines to more reliably model ALS, a fatal neurodegenerative disease.

One study, the results of which were published in *Cell*, announced the creation of a genomic “scorecard,” designed for a comprehensive characterization of pluripotent cell lines. Led by Alexander Meissner and Christoph Bock, the team used genomic methods to compare and characterize 20 ES cell lines and 12 iPS cell lines. The scorecard will allow scientists everywhere to optimize and streamline the selection and monitoring of pluripotent cell lines.

The results of a complementary study appeared online in *Nature Biotechnology*. Kevin Eggan, Gabriella Boutling et al. authored the study, a rigorous and extensive characterization of 16 iPS cell lines. All 16 lines passed a stringent test of differentiation capacity. This thorough test will serve as an additional resource for all those interested in the application of pluripotent stem cells to human disease.

“Our team has created a toolbox that allows us to test the potential of stem cell lines created from a tiny sample of ALS patient skin,” said Valerie Estess, director of research for Project A.L.S. “In fact, the Project A.L.S./Jenifer Estess Lab is already using these well defined ALS patient lines not only to model ALS, but to screen for drugs that may slow or stop the human disease.”

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Project A.L.S.TM is the new paradigm for brain disease research. Its mission is to recruit the world’s finest scientists and clinicians to work together rationally and aggressively toward an understanding of and the first effective treatments for ALS (amyotrophic lateral sclerosis), also known as Lou Gehrig’s disease. The Project A.L.S./Jenifer Estess Laboratory for Stem Cell Research, in New York, is the world’s first and only privately funded laboratory to work exclusively on stem cells and ALS.

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