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## **California Stem Cell and ALS Therapy Development Institute Extend Their Collaboration to Advance Potential Stem Cell Assisted Therapy for ALS**

*Collaboration will further evaluate novel stem cell-derived product for the treatment of fatal neurodegenerative disease by regulating genes relevant to disease progression*

CAMBRIDGE, Mass. & IRVINE, Calif. – February 2, 2010 – California Stem Cell, Inc. (CSC) and ALS Therapy Development Institute (ALS TDI) are pleased to announce an extension and expansion of their collaboration aimed at advancing a potential stem cell therapy for ALS (amyotrophic lateral sclerosis). This effort will build on work that has already been completed as part of this on-going partnership to understand how stem cells, and their derivatives, may be applied to treatment of this fatal neurodegenerative disease.

In this round of experiments, ALS TDI will use a CSC high-purity line of stem cells, called MotorGraft™, to deliver gene expression modifying payloads to specific locations within the central nervous system. The Institute's "Knowledgesphere", a database of information on gene and protein expression changes related to disease, contains several gene candidates to transfect into stem cells and deliver into the body. The initial phase of this new collaboration will confirm the expression of these genes *in vivo*. Once expression is confirmed, the collaborators will initiate the second phase of this new collaboration, which calls for several full efficacy experiments in order to measure for any potential ameliorating disease. Members of the CSC science team will travel to ALS TDI to administer the stem cells as part of each experiment.

"As part of our mission, we feel it prudent to work in this arena and will do our part to help advance the understanding of how stem cells could be used to aid in the slowing or stopping of ALS disease progression. We were encouraged by the previous round of experiments with California Stem Cell and are excited to build on the knowledge gained," said Steve Perrin, Ph.D., chief executive officer and chief scientific officer of ALS TDI.

The two groups previously worked together to conduct experiments aimed at transplanting high purity human stem cell progenitor motor neurons, into the spinal cords of SOD1 mice, which are genetically engineered to exhibit the neuronal deterioration and mimic ALS. The results of those studies showed that the cells survive, integrate, and display markers of motor neurons when transplanted into the SOD1 mouse model.

"ALS TDI's expertise in ALS disease biology and their world-class screening facility make them an ideal partner to collaborate with in order to understand the use and benefit of our MotorGraft™ technology in treating ALS. We know we have the most stable and pure population of stem cell derived motor neuron progenitors available in the world, and are excited to see if it can be used to help fight this terrible disease," said Chris Airriess, chief operating office for California Stem Cell.

ALS, also known as Lou Gehrig's disease, is a neurodegenerative disease resulting in progressive paralysis and is considered fatal. The disease strikes typically without an identifiable cause, indiscriminately affecting a new family every 90 minutes in the U.S. That incidence rate is similar to that of multiple sclerosis, but the typical survival prognosis given to a new ALS patient is only 2-5 years from their date of diagnosis. Currently, there is no known cure for ALS and only one FDA-approved drug, with marginal efficacy, for treating the disease.

California Stem Cell recently presented the findings of previous work done with ALS TDI to the FDA, as part of an a Pre-Investigational New Drug meeting to discuss the potential of CSC's MotorGraft™ to treat another degenerative disease, Spinal Muscular Atrophy (SMA). This supports the use of this therapeutic product to treat multiple disorders, such as SMA, ALS, spinal cord injury and transverse myelitis.

#### **About ALS Therapy Development Institute:**

The mission of the ALS Therapy Development Institute (ALS TDI) is to develop effective therapeutics that slow or stop amyotrophic lateral sclerosis (ALS, Lou Gehrig's disease), as soon as possible. Focused on meeting this urgent unmet medical need, ALS TDI executes a robust discovery program, as well as a multi-pronged approach to validate potential therapeutics; including small molecules, protein biologics, gene therapies and cell-based constructs. The world's first non-profit biotech, ALS TDI has developed an industrial-scale platform that allows for the development and testing of dozens of potential therapeutics each year. Built by and for patients, the Institute is the world's only non-profit biotechnology company with more than 30 professional scientists. In addition, the Cambridge, Massachusetts based research Institute collaborates with leaders in both academia and industry. For more information, please visit us online at [www.als.net](http://www.als.net).

#### **About California Stem Cell, Inc:**

California Stem Cell, Inc is a privately held company focused on the manufacturing of high-purity human cells for conventional drug screening and clinical application. Since its founding in 2005, CSC has developed and has intellectual property surrounding methods for scalable production of human motor neurons, neuronal progenitors, cardiomyocyte progenitors and hepatocytes cells at its Irvine, California facility. CSC is currently in the pre-clinical development stage of stem cell-derived therapies for spinal muscular atrophy (SMA), ALS and spinal cord injury.

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