

**Families of Spinal Muscular Atrophy; University of California, Irvine Stem Cell Scientist; and California Stem Cell Announce Collaboration for Safety Studies for Stem Cell Therapy in Spinal Muscular Atrophy**

**Safety Studies Leading to FDA Application for Stem Cell Therapy in SMA will begin in early 2008**

*February 19, 2008:* Families of Spinal Muscular Atrophy (FSMA); a stem cell scientist at University of California, Irvine (UCI); and California Stem Cell, Inc. (CSC) are pleased to announce a partnership to advance a potential stem cell therapy for SMA to human clinical trials. The specific set of animal studies planned, which will be conducted in accordance with FDA regulations, will assess the safety of motor neuron progenitors derived from human stem cells after transplantation.

SMA is a genetic disorder with no current treatment that is the leading genetic killer of children younger than two years of age. SMA typically is marked by the degeneration of voluntary muscle movement including the muscles that control crawling, walking, swallowing or breathing due to the dysfunction or death of motor neurons. It is a debilitating and often fatal disease.

These safety studies are the critical steps in advancing stem cell therapy into human trials for SMA. High purity human motor neuron populations for use in transplant therapies were developed by CSC and have been used successfully in proof of concept efficacy and preliminary safety studies in the laboratory of Dr. Hans Keirstead at UCI with funding from FSMA. CSC employs scalable manufacturing protocols to produce and supply the large population of clinical-grade motor neuron progenitors required for these pivotal studies and future human clinical trials.

“This collaboration illustrates the breadth of skills that are required to take a potential treatment from the bench to the bedside. I am confident that we have assembled the right team and the right plan to move this treatment forward with both diligence and speed,” said Hans S. Keirstead, Professor, UCI.

“California Stem Cell is very pleased to be part of this collaboration with Families of SMA and UCI and optimistic, based on preliminary data, about the very real potential for success in developing a cell transplant therapy for SMA, which devastates so many families.” said Chris Airriess, Chief Operating Officer, CSC.

Studies are being conducted in parallel at both the Keirstead laboratory at UCI and the laboratory of Dr. Douglas Kerr at Johns Hopkins University, with funding from FSMA, to show the benefit of human motor neuron progenitor replacement in animal models of motor neuron disease. This builds on the pioneering work published by Dr. Kerr in the *Annals of Neurology* in 2005 showing that transplants of mouse embryonic stem cell-derived motor neurons into the spinal cord can connect with muscles and partially restore function in paralyzed rats.

“This program holds a tremendous amount of promise for our patients and families. While FSMA makes significant investments in traditional and relatively low-risk drug approaches to find a treatment for SMA, alternative therapies like stem cells may hold the key to a cure. Our strategy is to fund both approaches in parallel to reach our goals as quickly as possible while minimizing risks” said Kenneth Hobby, Executive Director, FSMA. “Families of SMA is extremely grateful to The Dhont Family Foundation for the support of \$200,000 given to help move this stem cell program forward.”

Stem cell therapy for SMA has the potential to replace the motor neurons lost during the disease course. While other types of therapies have the potential to slow disease progression and possibly increase

strength, motor neuron replacement through the use of stem cells is the only means to replace motor neurons once they are gone.

This strategy may be useful for treating multiple disorders such as spinal cord injury, transverse myelitis, and Amyotrophic Lateral Sclerosis (ALS) in addition to Spinal Muscular Atrophy.

### **About Families of Spinal Muscular Atrophy:**

Families of SMA is dedicated to developing a treatment and cure for SMA by promoting and supporting research, helping families cope through informational programs and support, and educating the public and the medical community about SMA. The organization, originally founded in 1984 by a small group of parents, has grown to more than 32 chapters and affiliates worldwide and more than 50,000 supporters. FSMA receives the majority of its revenues through volunteer efforts, funding almost \$40 million to date for research. FSMA-sponsored research has made significant contributions to better understanding SMA and advancing new therapies towards human clinical testing. See [www.curesma.org](http://www.curesma.org) for more information. Media contact: [info@fsma.org](mailto:info@fsma.org) or call 800 886-1762.

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

Irvine, CA based California Stem Cell, Inc is a privately held company focused on the manufacturing of high purity human cells for therapeutic development and clinical application. Founded in 2005, CSC has developed proprietary methods for scalable production of human motor neurons, neuronal progenitors, cardiac muscle and sino-atrial node cells. Media contact: [press@californiastemcell.com](mailto:press@californiastemcell.com)

### **About the University of California, Irvine:**

The University of California, Irvine is a top-ranked university dedicated to research, scholarship and community service. Founded in 1965, UCI is among the fastest-growing University of California campuses, with

more than 27,000 undergraduate and graduate students, and nearly 2,000 faculty members. The third-largest employer in dynamic Orange County, UCI contributes an annual economic impact of \$3.6 billion. For more UCI news, visit [www.today.uci.edu](http://www.today.uci.edu).