
Human Embryonic Stem Cell-Derived Neural Stem Cells for Severe Spinal Cord Injury (SCI)

Grant Award Details

Human Embryonic Stem Cell-Derived Neural Stem Cells for Severe Spinal Cord Injury (SCI)

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-11579

Project Objective: To complete activities supporting the development of the GMP-compatible therapeutic candidate, H9 ESC derived human neural stem cells in a gel-like matrix for the treatment of spinal cord injury, and to conduct a well prepared pre-IND interaction with the FDA resulting in correspondence from the FDA confirming agreement with the IND-enabling preclinical plan.

Investigator:

Name:	Mark Tuszynski
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Neurological Disorders, Spinal Cord Injury

Human Stem Cell Use: Embryonic Stem Cell

Award Value: \$6,235,897

Status: Active

Grant Application Details

Application Title: Human Embryonic Stem Cell-Derived Neural Stem Cells for Severe Spinal Cord Injury (SCI)

Public Abstract:**Translational Candidate**

H9 (WA09) embryonic stem cell-derived neural stem cells with a spinal cord identity (H9-NSCsc)

Area of Impact

Severe spinal cord injury

Mechanism of Action

Our candidate therapy for SCI uses human neural stem cells in a gel-like matrix containing growth factors. We aim to fill the injury site with replacement neural stem cells that can form new neural "relays" across the injury to restore function. This approach may potentially treat severe SCI by repairing injured connections, in contrast to other stem cell clinical trials for SCI that only aim to improve the function of axons that are spared by the injury.

Unmet Medical Need

20,000 Americans sustain SCI each year, and more than 300,000 live with chronic injury, extracting a huge physical, emotional and financial toll. There are no therapies to repair the spinal cord. We aim to regenerate the injured spinal cord by "splicing" neural circuits, thereby restoring function.

Project Objective

Pre-IND meeting

Major Proposed Activities

- Generate GMP-compliant H9 ESC Master and Working cell banks (MCB, WCB), as well as GMP-compatible H9-NSCsc MCB and WCBs.
- Rodent studies to establish proof of concept and pilot safety.
- Develop Chemistry, Manufacturing, and Control (CMC) characterization and release assays for the candidate H9-NSCsc.

Statement of Benefit to California:

SCI affects approximately 300,000 people in the U.S., with more than 20,000 new injuries per year. People with SCI often endure decades of severe disability, with staggering physical, emotional, and financial costs. The first year of treatment alone is \$1 million for a quadriplegic patient. Better treatments are needed, and even a modest increase in functional capacity (1-2 spinal levels) can produce meaningful improvement in quality of life and cost savings for California.

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