A Phase I/IIa Dose Escalation Safety Study of AST-OPC1 in Patients with Cervical Sensorimotor Complete Spinal Cord Injury

Grant Award Details

A Phase I/IIa Dose Escalation Safety Study of AST-OPC1 in Patients with Cervical Sensorimotor Complete Spinal Cord Injury

Grant Type: Strategic Partnership III Track A

Grant Number: SP3A-07552

Project Objective: Conduct a phase I/IIa Dose Escalation Safety Study of AST-OPC1 cells in patients with Cervical Sensorimotor Complete Spinal Cord Injury.

Investigator:

Name:	Francois Binette
Institution:	Lineage Cell Therapeutics Inc.
Type:	PI

Disease Focus: Neurological Disorders, Spinal Cord Injury

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $14,323,318

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Operational Milestone #A

View Report

Reporting Period: Operational Milestone OM#B

View Report

Reporting Period: Operation Milestone OM: #D

View Report

Grant Application Details

Application Title: A Phase I/IIa Dose Escalation Safety Study of [REDACTED] in Patients with Cervical Sensorimotor Complete Spinal Cord Injury

Public Abstract: The proposed project is designed to assess the safety and preliminary activity of escalating doses of human embryonic stem cell derived oligodendrocyte progenitor cells (OPCs) for the treatment of spinal cord injury. OPCs have two important functions: they produce factors which stimulate the survival and growth of nerve cells after injury, and they mature in the spinal cord to produce myelin, the insulation which enables electrical signals to be conducted within the spinal cord.

Clinical testing of this product initiated in 2010 after extensive safety and efficacy testing in more than 20 nonclinical studies. Initial clinical safety testing was conducted in five subjects with neurologically complete thoracic injuries. No safety concerns have been observed after following these five subjects for more than two years. The current project proposes to extend testing to subjects with neurologically complete cervical injuries, the intended population for further clinical development, and the population considered most likely to benefit from the therapy. Initial safety testing will be performed in three subjects at a low dose level, with subsequent groups of five subjects at higher doses bracketing the range believed most likely to result in functional improvements. Subjects will be monitored both for evidence of safety issues and for signs of neurological improvement using a variety of neurological, imaging and laboratory assessments.

By completion of the project, we expect to have accumulated sufficient safety and dosing data to support initiation of an expanded efficacy study of a single selected dose in the intended clinical target population.

Statement of Benefit to California:

The proposed project has the potential to benefit the state of California by improving medical outcomes for California residents with spinal cord injuries (SCIs), building on California's leadership position in the field of stem cell research, and creating high quality biotechnology jobs for Californians.

Over 12,000 Americans suffer an SCI each year, and approximately 1.3 million people in the United States are estimated to be living with a spinal cord injury. Although specific estimates for the state of California are not available, the majority of SCI result from motor vehicle accidents, falls, acts of violence, and recreational sporting activities, all of which are common in California. Thus, the annual incidence of SCI in California is likely equal to or higher than the 1,400 cases predicted by a purely population-based distribution of the nationwide incidence.

The medical, societal and economic burden of SCI is extraordinarily high. Traumatic SCI most commonly impacts individuals in their 20s and 30s, resulting in a high-level of permanent disability in young and previously healthy individuals. At one year post injury, only 11.8% of SCI patients are employed, and fewer than 35% are employed even at more than twenty years post-injury (NSCISC Spinal Cord Injury Facts and Figures 2013). Life expectancies of SCI patients are significantly below those of similar aged patients with no SCI. Additionally, many patients require help with activities of daily living such as feeding and bathing. As a result, the lifetime cost of care for SCI patients are enormous; a recent paper (Cao et al 2009) estimated lifetime costs of care for a patient obtaining a cervical SCI (the population to be enrolled in this study) at age 25 at $4.2 million. Even partial correction of any of the debilitating consequences of SCI could enhance activities of daily living, increase employment, and decrease reliance on attendant and medical care, resulting in substantial improvements in both quality of life and cost of care for SCI patients.

California has a history of leadership both in biotechnology and in stem cell research. The product described in this application was invented in California, and has already undergone safety testing in five patients in a clinical study initiated by a California corporation. The applicant, who has licensed this product from its original developer and recruited many of the employees responsible for its previous development, currently employs 17 full-time employees at its California headquarters, with plans to significantly increase in size over the coming years. The successful performance of the proposed project would enable significant additional jobs creation in preparation for pivotal trials and product registration.
