

## Phenotypic Analysis of Human ES Cell-Derived Muscle Stem Cells

### Grant Award Details

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Phenotypic Analysis of Human ES Cell-Derived Muscle Stem Cells

**Grant Type:** Basic Biology III

**Grant Number:** RB3-05041

**Project Objective:** The overall goal of this project is to use marker hESC lines to define the cellular and functional phenotypes of human muscle stem cells as they differentiate along the muscle lineage and to determine the best source for transplant.

**Investigator:**

<b>Name:</b>	Jason Pomerantz
<b>Institution:</b>	University of California, San Francisco
<b>Type:</b>	PI

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**Disease Focus:** Muscular Dystrophy, Skeletal/Smooth Muscle disorders

**Human Stem Cell Use:** Embryonic Stem Cell

**Award Value:** \$1,381,296

**Status:** Closed

### Progress Reports

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**Reporting Period:** Year 1

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**Reporting Period:** Year 2

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**Reporting Period:** Year 3

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**Reporting Period:** NCE (Year 4)

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## Grant Application Details

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**Application Title:** Phenotypic Analysis of Human ES Cell-Derived Muscle Stem Cells

**Public Abstract:** We study human muscle development, and are actively investigating potential cell-based therapies for the treatment of degenerative muscle diseases, such as muscle dystrophy. This project will define the pathway that muscle stem cells follow as they form new muscle, and identify which muscle stem cells are most useful for therapy. Our approach will be to examine human embryonic stem cells as they become muscle stem cells and mature muscle in culture, to define the stages of normal muscle development. We will then transplant these stem cells at various stages of development into the leg muscles of mice with muscular dystrophy, and study how these cells become new muscle tissue, how this impacts the animals' ability to exercise, and the strength of the treated muscles. Our goal for this research is to fully understand the normal process of human muscle stem cell development, and to identify specific stem cells that provide therapeutic benefit when transplanted into dystrophic muscle.

**Statement of Benefit to California:** Muscular dystrophies are profoundly debilitating disorders that affect more than 1 in 3,500 male births. They comprise a group of genetic diseases that cause progressive weakness and damage to skeletal muscle resulting from abnormal proteins critical to muscle health. These abnormal proteins are thought to predispose muscle to damage from normal activity, leading to premature depletion of normal muscle stem cells that maintain muscle health during normal use. This research will identify human embryonic stem cells that are able to repair damaged muscle, thereby providing a new approach to therapy for patients with muscle disease. The medical treatments developed as a result of these studies will not only benefit the health of Californians with muscular dystrophy and other degenerative muscle diseases, but also should result in significant savings in health care costs. This research will push the field of muscle regenerative medicine forward despite the paucity of federal funds for embryonic stem cell research, and better prepare us to utilize these funds when they become available in the future.

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