A Double-Blind Randomized Placebo-Controlled Investigation of Autologous Muscle Derived Progenitor Cells for the Treatment of Dysphagia

Grant Award Details

A Double-Blind Randomized Placebo-Controlled Investigation of Autologous Muscle Derived Progenitor Cells for the Treatment of Dysphagia

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-13017

Project Objective: The overall objective of this project is to complete a double-blind randomized placebo-controlled Phase 1/2 trial of Autologous Muscle Derived Progenitor Cells (ADMC) for the treatment of dysphagia. The primary activities will include enrollment of 62 patients to be treated with two doses of ADMC, administered 4-6 weeks apart, that will be injected into the tongue for the purpose of remuscularization, as well as two years of follow-up visits and writing of a clinical study report. The manufacturing of the autologous product from a leg muscle biopsy for each patient will be done by Cook Myosite, and will not be funded by CIRM.

Investigator:

Name:	Peter Belafsky
Institution:	University of California, Davis
Type:	PI

Disease Focus: Muscle Injury, Skeletal/Smooth Muscle disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: $11,015,936

Status: Active

Grant Application Details

Application Title: A Double-Blind Randomized Placebo-Controlled Investigation of Autologous Muscle Derived Progenitor Cells for the Treatment of Dysphagia

Public Abstract:

Therapeutic Candidate or Device

Autologous Muscle Derived Progenitor Cells (AMDC) isolated from skeletal muscle biopsy

Indication

Subjects with dysphagia (swallowing difficulties) that develops following treatment for head and neck cancer

Therapeutic Mechanism

Autologous Muscle Derived Progenitor Cells differentiate to form new muscle fibers, engraft into existing myofibers, and have been shown to increase muscle diameter and function. This proposed trial will target tongue musculature, a critical component of a safe and efficient swallow. Restoration of tongue muscle function will improve swallowing outcomes for patients with dysphagia following head and neck cancer.

Unmet Medical Need

Consequences of dysphagia include malnutrition, dehydration, social isolation, feeding tube dependency, depression, aspiration pneumonia, pulmonary abscess, and death. Despite the devastating consequences caused by treatment for HNC, few effective therapeutic options exist.

Project Objective

Phase I/II completed

Major Proposed Activities

- Manufacture 62 AMDC products to supply the trial
- Administer two doses of AMDCs to 31 subjects and two doses placebo to 31 additional subjects
- Assess safety and efficacy of AMDC for for the treatment of tongue dysphagia (TD) that develops following treatment for head and neck cancer

Statement of Benefit to California:

The results of this clinical trial will directly benefit millions of underserved Californians with swallowing disorders who currently have no effective treatment. If successful, we anticipate greatly accelerated commercialization of our product candidate to meet this unmet clinical need. The results of this investigation will have far-reaching implications for the use of AMDCs in the treatment of other vulnerable populations with dysphagia and individuals with muscle injury from other causes.

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