
Embryonic Stem Cell-Derived Chondroprogenitor Cells to Repair Osteochondral Defects

Grant Award Details

Embryonic Stem Cell-Derived Chondroprogenitor Cells to Repair Osteochondral Defects

Grant Type: Preclinical Development Awards

Grant Number: PC1-08128

Project Objective: The goal of this project is to have a successful pre-IND meeting with the FDA after conducting combined safety, efficacy, dosing and tumorigenicity studies with the development candidate (hESC-derived chondroprogenitors) seeded into a hydrogel matrix consisting of Collagen Type 2, Hyaluronic Acid, and GMP grade Master Cell Banks for the hESC and the hESC-derived chondroprogenitors will be made.

Investigator:

| | |
|---------------------|----------------|
| Name: | Darryl D'Lima |
| Institution: | Scripps Health |
| Type: | PI |

Disease Focus: Bone or Cartilage Disease

Human Stem Cell Use: Embryonic Stem Cell

Award Value: \$7,660,211

Status: Closed

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Embryonic Stem Cell-Derived Chondroprogenitor Cells to Repair Osteochondral Defects

Public Abstract:

Surgical approaches to the treatment of focal cartilage defects can be classified into repair, replacement, and regeneration therapies. Marrow stimulation procedures such as microfracture result in a repair tissue that is predominantly fibrocartilaginous in nature, which is mechanically less durable than articular cartilage and survives on average 7 years before requiring another procedure. Osteochondral grafting (autologous or allogeneic) replaces the defect with fresh mature cartilage and bone. While the tissue replicates natural cartilage, the grafted cartilage does not integrate or bond with the host tissue. Autologous chondrocyte implantation (ACI) attempts to regenerate tissue by injecting chondrocytes into the defect. Results with this technique are mixed with several randomized clinical trials failing to find a clinically and statistically significant benefit over microfracture or other procedures.

Our approach is to advance third-generation cell therapy by constructing scaffolds that are seeded with chondroprogenitor cells programmed to undergo differentiation into bone and cartilage cells. If successful, this will be the first-in-man embryonic stem-cell-based treatment of an orthopaedic disease that has challenged repeated attempts over the last 400 years. The product has the unique advantage that the same material is universally applicable in all patients with a range of different defect shapes and sizes. The preclinical development, characterization, efficacy, and safety will also support and advance stem-cell-based regenerative medicine in general.

Statement of Benefit to California:

Arthritis is a common disease and increases with age. The annual cost of treating arthritis in the US is estimated to be over \$200B in 2013. Over a million joint replacements are performed in the US alone for end-stage arthritis. However, for younger patients with severe arthritis or impending arthritis there is as yet no treatment that can prevent, cure, or even slow the progression of this disease. In this proposal, we target bone and cartilage defects that are a major factor in contributing to early osteoarthritis in patients less than 55 years of age. Our approach is to advance third-generation cell therapy by constructing scaffolds that are seeded with chondroprogenitor cells programmed to undergo differentiation into bone and cartilage cells. This proposal falls under the mission statement of CIRM for funding innovative research. A stem-cell-based approach for treating articular cartilage defects is not represented in CIRM's current portfolio. If successful, this will be the first-in-man embryonic stem-cell-based treatment of an orthopaedic disease that has challenged repeated attempts over the last 400 years. This will further validate the significance of the CIRM program and help maintain California's leading position at the cutting edge of biomedical research.

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