Pluripotent stem cell-derived chondrocytes for articular cartilage repair

Grant Award Details

Pluripotent stem cell-derived chondrocytes for articular cartilage repair

Grant Type: Therapeutic Translational Research Projects
Grant Number: TRAN1-09288
Project Objective: Pre-IND Meeting with the FDA

Investigator:

<table>
<thead>
<tr>
<th>Name</th>
<th>Denis Evseenko</th>
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<tr>
<td>Institution</td>
<td>University of Southern California</td>
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<td>Type</td>
<td>PI</td>
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Disease Focus: Bone or Cartilage Disease

Human Stem Cell Use: Embryonic Stem Cell

Award Value: $2,503,104

Status: Active

Grant Application Details

Application Title: Pluripotent stem cell-derived chondrocytes for articular cartilage repair
Public Abstract: **Translational Candidate**

We propose to develop a universal, off-the-shelf treatment for articular cartilage repair based on pluripotent stem cell (PSC)-derived chondrocytes

**Area of Impact**

The proposed therapy could treat the major cartilage lesions present in more than 10% of people under 50, which often result in pain and arthritis

**Mechanism of Action**

Untreated cartilage defects often lead to joint pain and degeneration over time, often requiring joint replacement. The proposed candidate is a universal cell therapy designed to generate new articular cartilage in these defects and interrupt the cycle of degeneration.

**Unmet Medical Need**

Approximately 10% of people under 50 have at least a single, high-grade defect in their knee cartilage; these lesions have a high probability of promoting subsequent degenerative processes, often resulting in arthritis. Currently, there are no effective treatments for altering this progression.

**Project Objective**

Pre-IND meeting

**Major Proposed Activities**

- Optimization of large-scale production, preservation and quality control of pluripotent stem cell-derived chondrocytes
- Conduct nonclinical safety and stability tests in small and large animal models
- Develop a draft clinical protocol and synopsis and conduct a successful pre-IND meeting

**Statement of Benefit to California:**

The work described in this proposal is designed to produce a universal treatment for articular cartilage lesions. If successfully validated, this cellular therapy will likely help reduce joint pain and reduce further degeneration of joints that leads to arthritis. The proposed treatment may be of major public benefit, as it would represent the first curative strategy for cartilage injury and subsequent degeneration, likely decreasing economic burden on the state and its people.

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