Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis

Grant Award Details

Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis

Grant Type: Late Stage Preclinical Projects
Grant Number: CLIN1-08309

Project Objective: To develop a small molecule treatment for osteoarthritis injected intra-articularly to enhance MSC chondrogenic differentiation in vivo. This award is running in parallel with the team's PC1 award. This CLIN1 award funds the GMP manufacturing of the drug and the conduct of the IND-enabling toxicology studies to complete the IND package and file an IND with the FDA for a Phase 1 trial.

Investigator:

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<tr>
<th>Name</th>
<th>Peter Schultz</th>
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<td>Institution</td>
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Disease Focus: Bone or Cartilage Disease
Award Value: $1,667,832
Status: Closed

Progress Reports

- Reporting Period: Operational Milestone (OM) #1
  View Report
- Reporting Period: Final Operational Milestone #2
  View Report

Grant Application Details

Application Title: Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis
Therapeutic Candidate or Device

The therapeutic candidate is a drug-like small molecule that promotes cartilage resident mesenchymal stem cell differentiation into chondrocytes.

Indication

Osteoarthritis and cartilage injury

Therapeutic Mechanism

The therapeutic candidate, through intra-articular administration, promotes cartilage resident mesenchymal stem cell differentiation into chondrocytes. The newly formed chondrocytes replace the dead chondrocytes, synthesize and secrete extracellular matrix proteins, which leads to the repair of damaged cartilage in OA patients or following traumatic injury.

Unmet Medical Need

Current therapeutic options for OA are limited to pain or symptom-modifying drugs and joint replacement surgery; no disease-modifying drugs are approved for clinical use. The therapeutic candidate, if successful, will be the first-in-class regenerative medicine for OA and cartilage injury.

Project Objective

IND filing and initiating Phase 1 clinical trial

Major Proposed Activities

- IND document preparation and filing
- GLP toxicology and safety profiling of the therapeutic candidate
- Non-GLP determination of maximum tolerated doses upon local administration

Statement of Benefit to California:

Osteoarthritis (OA) is the most prevalent musculoskeletal disease and globally the 4th leading cause of Years Lost to Disease (YLD). The annual economic impact of arthritis in the U.S. is estimated at over $100 billion. No disease-modifying OA drugs are approved for clinical use. Clearly, the development of a new disease-modifying therapeutic would have a significant impact on the well-being of Californians and reduce the negative economic impact on the state.

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