
An autologous somatic stem cell therapy for the treatment of osteonecrosis

Grant Award Details

An autologous somatic stem cell therapy for the treatment of osteonecrosis

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-09270

Project Objective: To demonstrate scalable manufacture of an autologous somatic stem cell therapy for the treatment of osteonecrosis allowing for the conduct of a well-prepared pre-IND meeting.

Investigator:

Name:	Ying Zhu
Institution:	Ankasa Regenerative Therapeutics
Type:	PI

Disease Focus: Bone or Cartilage Disease, Osteonecrosis

Human Stem Cell Use: Adult Stem Cell

Award Value: \$2,088,780

Status: Closed

Progress Reports

Reporting Period: Final Operational Milestone #2

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Grant Application Details

Application Title: An autologous somatic stem cell therapy for the treatment of osteonecrosis

Public Abstract:**Translational Candidate**

An autologous somatic stem cell therapy for the treatment of osteonecrosis.

Area of Impact

Osteonecrosis is a painful, progressive disease for which there is no treatment, save replacing the dead bone with a metal implant.

Mechanism of Action

Autografts contain skeletal stem cells. In young patients, these stem cells differentiate and give rise to new bone but in older patients, autografts are ineffective. The WNT therapeutic ART352-L re-activates stem cells in an older person's autograft and the resulting material, ART1001, generates more osteo-progenitor cells and engrafts better than untreated autografts. In preclinical models ART1001 outperforms the standard of care and leads to superior healing of osteonecrotic lesions.

Unmet Medical Need

Osteonecrosis is a disease that "causes jawbones to rot and thighbones to snap", and its incidence is on the rise in our aging population. The autologous somatic stem cell therapy ART1001 has the potential to generate more bone, sooner in these older patients with osteonecrotic lesions.

Project Objective

Conduct of a well prepared pre-IND meeting.

Major Proposed Activities

- ART352 process development; development of liposomal formulation (e.g., ART352-L); GLP production of ART352 and ART352-L; stability studies.
- Evaluate safety of the autologous stem cell product ART1001; validate method of ART1001 delivery in a large animal model
- Determine clinical plan and regulatory pathway for ART1001; schedule and conduct a pre-IND meeting with the FDA.

Statement of Benefit to California:

For Californians over 45, low bone mass diseases are a major public health threat: They account for more days spent in hospital than diabetes and heart attacks, and their related disabilities are greater than those caused by cancers. ART1001 has the potential to dramatically improve bone healing in this older population. Such an improvement in the SOC will result in better outcomes, fewer complications, and a quicker return of older individuals back to the activities of daily living.

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