

Osteoporosis, Bone and Cartilage Disease Fact Sheet

CIRM funds many projects seeking to better understand bone related diseases including osteoporosis, osteoarthritis and osteonecrosis to translate those discoveries into new therapies.

Description

Estimates of the number of people in the U.S. with osteoporosis range from 10 million to 25 million, with 75 percent being women. For many of those individuals it can be a disease with minimal immediate impact but incredible lingering risk. Between 1.5 million and 2 million of those with the condition develop osteoporosis-related fractures each year. About 70 percent of fractures are in the vertebra of the spine, and they can range from minor to completely debilitating. The next most common fracture is in the hip, which increases the risk of premature death and frequently lands otherwise healthy functional elderly in nursing homes for the remainder of their lives. Osteoporosis costs the nation an estimated \$19 billion a year.

Osteoarthritis is a disease that affects the cartilage in joints. It is one of the most common forms of disability, effecting more than 27 million people in the U.S. CIRM funds several projects looking to replace or repair the cartilage lost in the degenerative disease. Projects include creating new cartilage from donor stem cells as well as developing a drug to drive a person's own stem cells to do a better job of repair.

Osteonecrosis is a disease that decreases blood circulation to bones causing them to weaken and eventually die. If left untreated, patients with osteonecrosis can develop end-stage hip arthritis and require surgical joint replacement. Despite the low prevalence compared to primary osteoarthritis or degenerative arthritis, femoral head (hip) osteonecrosis has a significant economic impact because it largely affects individuals in the prime of life (peak age 35 years).

CIRM also funds project to help the thousands who live with fractures too big to heal or fractures in older individuals that their aging bodies can no longer heal. Between the ages of 30 and 80 we have a 10-fold decrease in the number of these stem cells and the ones we have left are less effective at replacing and repairing bone. California's stem cell agency has funded several projects that propose various ways to increase the number of these stem cells, or improve their effectiveness to help keep bones healthier longer (the full list of CIRM awards is below).

Clinical Stage Programs

California Institute for Biomedical Research

Researchers at the California Institute for Biomedical Research (CALIBR) have been awarded \$8.447 million to test KA34, a drug that, in preclinical tests, recruits stem cells to create new cartilage in areas damaged by osteoarthritis. CIRM funded the research that developed this technology and now this Phase 1 trial will test this stem cell directed treatment in people with osteoarthritis of the knee, hopefully slowing down or even halting the progression of the disease.

- [Learn more about this clinical award](#)
- [Learn more about this trial on clinicaltrials.gov](#)

UC Davis

A team at UC Davis has developed a new stem cell-based treatment for osteonecrosis. They are using a small molecule drug that targets the mesenchymal stem cells in a patient's bone marrow and directs the stem cells to the surface of the bone where they then develop new bone tissue. The team is testing the safety and efficacy of this drug treatment in a Phase 1 clinical trial.

- [Learn more about this clinical award](#)
- [Learn more about this trial on clinicaltrials.gov](#)

CIRM Grants Targeting Bone & Cartilage Disease

Researcher name	Institution	Grant Title	Grant Type	Award Amount
Peter Schultz	Calibr	Development of a Chondrogenic Drug Candidate Targeting Cartilage-residing Mesenchymal Stem Cells for the Treatment of Osteoarthritis	Preclinical Development Awards	\$2,306,703
Peter Schultz	Scripps Research Institute	Cartilage Regeneration by the Chondrogenic Small Molecule PRO1 during Osteoarthritis	Early Translational II	\$6,047,249
Darryl D'Lima	Scripps Health	Embryonic Stem Cell-Derived Chondroprogenitor Cells to Repair Osteochondral Defects	Preclinical Development Awards	\$7,660,211
Bruno Peault	University of California, Los Angeles	Harnessing native fat-residing stem cells for bone regeneration	Early Translational II	\$5,359,076
Peter Schultz	Calibr	Development of a Chondrogenic Drug Candidate Targeting Resident Mesenchymal Stem Cells for the Treatment of Osteoarthritis	Late Stage Preclinical Projects	\$1,667,832
Dan Gazit	Cedars-Sinai Medical Center	Systemic Adult Stem Cell Therapy for Osteoporosis-Related Vertebral Compression Fractures	Early Translational II	\$1,927,698
A. Reddi	University of California, Davis	hESCs for Articular Cartilage Regeneration	SEED Grant	\$301,703
Gage DeKoeper Crump	University of Southern California	Skeletogenic Neural Crest Cells in Embryonic Development and Adult Regeneration of the Jaw	New Faculty II	\$2,247,403
Jack Wang	Cellular Biomedicine Group, Inc.	Allogenic human adipose-derived mesenchymal stem cells for the treatment of knee osteoarthritis	Late Stage Preclinical Projects	\$1,200,000
Darryl D'Lima	Scripps Health	Stem Cell-Based Therapy for Cartilage Regeneration and Osteoarthritis	Early Translational I	\$3,118,431
Kristen Johnson	Calibr	Evaluation of the Safety and Tolerability of KA34 in a Phase 1, Double-Blind, Dose Escalation Trial in Patients with Knee Osteoarthritis	Clinical Trial Stage Projects	\$8,447,523
Jill Helms	Stanford University	Enhancing healing via Wnt-protein mediated activation of endogenous stem cells	Early Translational I	\$6,464,126
Dmitriy Sheyn	Cedars-Sinai Medical Center	IVD rejuvenation using iPSC-derived notochordal cells	Inception - Discovery Stage Research Projects	\$241,992
Songtao Shi	University of Southern California	Oral and Craniofacial Reconstruction Using Mesenchymal Stem Cells	New Faculty I	\$3,242,651
Alireza Moshaverinia	University of California, Los Angeles	Gingival mesenchymal stem cells as a novel treatment modality for periodontal tissue regeneration	Inception - Discovery Stage Research Projects	\$194,483
Dan Gazit	Cedars-Sinai Medical Center	Genetically Engineered Mesenchymal Stem Cells for the Treatment of Vertebral Compression Fractures.	Disease Team Therapy Planning I	\$107,622

John Hood	Samumed, LLC	Clinical Development of an osteoinductive therapy to prevent osteoporosis-related fractures	Disease Team Therapy Planning I	\$99,110
Ying Zhu	Ankasa Regenerative Therapeutics	IND-enabling development of ART352-L, an endogenous stem cell reactivation therapy to enhance bone healing in the elderly	Late Stage Preclinical Projects	\$3,987,693
Nancy Lane	University of California, Davis	Increasing the endogenous mesenchymal stem cells to the bone surface to treat osteoporosis	Disease Team Therapy Planning I	\$107,750
Gayatri Rao	Rocket Pharmaceuticals, Inc.	A Phase I Clinical Trial for a Lentiviral Gene Therapy Targeting the TCIRG1 Gene for Infantile Malignant Osteopetrosis (IMO)	Clinical Trial Stage Projects	\$3,728,485
Kyriacos Athanasiou	University of California, Davis	Tissue engineered cartilage from autologous, dermis-isolated, adult, stem (DIAS) cells	Early Translational III	\$1,735,703
Frank Petrigliano	University of Southern California	Plurocart: a novel stem cell-based implant for articular cartilage restoration	Late Stage Preclinical Projects	\$5,999,782
Nancy Lane	University of California, Davis	Treatment of non-traumatic osteonecrosis with endogenous Mesenchymal stem cells	Disease Team Therapy Development - Research	\$18,231,356
Kevin Stone	The Stone Research Foundation for Sports Medicine and Arthritis	A Novel Therapy for Articular Cartilage Autologous Cellular Repair by Paste Grafting	Quest - Discovery Stage Research Projects	\$1,180,309
Dan Gazit	Cedars-Sinai Medical Center	Gene Targeting to Endogenous Stem Cells for Segmental Bone Fracture Healing	Early Translational IV	\$5,121,514
Darryl D'Lima	Scripps Health	Meniscal Repair and Regeneration	Quest - Discovery Stage Research Projects	\$1,361,775
J. Leach	University of California, Davis	Multi-modal technology for non-destructive characterization of bioengineered tissues	Tools and Technologies III	\$1,574,151
Fan Yang	Stanford University	Injectable Macroporous Matrices to Enhance Stem Cell Engraftment and Survival	Tools and Technologies III	\$1,434,235
Ying Zhu	Ankasa Regenerative Therapeutics	An autologous somatic stem cell therapy for the treatment of osteonecrosis	Therapeutic Translational Research Projects	\$2,088,780
Denis Evseenko	University of Southern California	Pluripotent stem cell-derived chondrocytes for articular cartilage repair	Therapeutic Translational Research Projects	\$2,503,104
Farhad Parhami	MAX BioPharma, Inc.	Therapeutic development of Oxy200, an oxysterol with bone anabolic and anti-resorptive properties for intervention in osteoporosis	Therapeutic Translational Research Projects	\$1,400,000
				Total: \$101,088,450.00



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#SciencePitch



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Resources

- CIRM blogs about bone & cartilage disease
- NIH Osteoporosis National Resource Center
- NIH Osteonecrosis Fact Sheet
- NIH Osteoarthritis Fact Sheet

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