UCI Sue & Bill Gross Stem Cell Research Center

UCLA UCI Alpha Stem Cell Clinic

The UCLA-UCI Alpha Stem Cell Clinic (ASCC) provides the necessary medical and operational expertise to effectively and efficiently drive novel stem cell therapies to clinical trials and ultimately change clinical practice. The UCI branch of the UCLA-UCI ASCC provides a centralized infrastructure from which to work with UCI faculty and staff to enhance stem cell clinical research and activities at UCI.

Message from Daniela A. Bota, MD, PhD

Associate Dean, Clinical Research; Vice Chair for Academic Affairs, Department of Neurology
Clinical Director, Sue and Bill Gross Stem Cell Research Center; Medial Director, Neuro-Oncology Program

As we find ourselves at the edge of 2018, I would like to take this opportunity to thank and congratulate you all on a progressive year as part of the UCLA-UCI Alpha Stem Cell Clinic (ASCC) UCI-based team. We have taken small steps and big leaps in the world of stem cell research. I encourage each of you to take a moment to reflect on your own personal growth and achievements, as well as those of the UCLA-UCI ASCC this past year.

In April, we embarked on a Phase 3 stem cell clinical trial to treat ALS patients. In June, one of own researchers, Magdalene J. Seiler, PhD, received $4.8 million from the California Institute for Regenerative Medicine (CIRM) as part of a grant to continue developing a stem cell-based therapy to treat vision loss. A portion of that grant also went toward the collaboration of the Sue & Bill Gross Stem Cell Research Center and AIVITA Biomedical to aid in the stem cell research. In August, we completed a double-blind, controlled Phase 2 trial, and are now studying the therapeutic effects of stem cells on patients with traumatic brain injuries. Throughout the year, we hosted and studied numerous other clinical trials.

It is because of the dedication and effort of the many individuals that make up our team that the UCLA-UCI ASCC has been able to lead stem cell research and clinical trials in California. For this, we are very proud.

In this edition of our UCI-based UCLA-UCI ASCC newsletter, we celebrate Suzanne Sandmeyer's addition to the CIRM Board, dive into the issues of dwindling funding and deceptive stem cell clinics, and share upcoming clinical trials and community lecture dates.

All the best,

DA Bota

Daniela A. Bota, MD, PhD

Suzanne Sandmeyer, PhD, Joins CIRM Board

Suzanne Sandmeyer, PhD, vice dean for Research at UCI School of Medicine, has been appointed to the board of the California Institute for Regenerative Medicine (CIRM). She was recommended for the position by UCI Chancellor Howard Gillman, who described her as "an outstanding researcher who has contributed significantly to the field of molecular genetics."

"It is a privilege to be involved in this new era of stem cell research and clinical trials," says Sandmeyer. "We have only just begun to understand the potential of our discoveries and the impact we can have on human health by advancing stem cell therapies."

Her research focuses on the molecular genetics and biochemistry of retrovirus-like elements called retrotransposons and on metabolic engineering in yeast.

When not making important scientific discoveries in the lab, you can find Sandmeyer pursuing one of her many and varied hobbies. "I go through phases like everyone. There is never enough time. My favorites are astronomy, bird photography, playing guitar, biking, kayaking, reading and, of course, German shepherd dogs."

Confronting Science Denialism, Deceptive Clinics and Dwindling Funding

In the early 2000s, Aileen Anderson, PhD, director of the UCI School of Medicine's Sue & Bill Gross Stem Cell Research Center, developed a useful transplant model for studying stem cells in the nervous system. Though Anderson didn't consider herself a stem cell scientist, colleagues began asking to run studies using her model. Now, she's a leading stem cell investigator in California.

Under Anderson, the center conducts clinical trials and studies how stem cells can benefit people with a variety of conditions. Its lab teams are working on "reprogramming" a body to not reject stem cells from a foreign source. They're hoping to tap the body's natural reserve of stem cells and repair organs from the inside.

Anderson recently won a $20,000 grant through UCI's Provost Initiative on Understanding and Engaging with Extremism, which will support a public outreach project on science denialism. It is a huge problem in the stem cell community; in part due to the growth of unregulated clinics that are selling unrealistic "cures" they claim to have derived from stem cells.

> "These clinics set their patients up with unrealistic expectations," Anderson says. "We want to inform people about what is and isn't currently medically possible."

Anderson's team devised a program involving an education forum for scientists, researchers and academics in the stem cell field, creating websites and distributing information so people can conduct research before receiving expensive, ineffective treatments.

Opening communication about stem cell research will be especially important over the coming years. The California Institute for Regenerative Medicine (CIRM), a $3-billion stem cell fund established through a 2004 ballot initiative, will soon run out of money. Supporters are now crafting a measure for the 2020 ballot that could give scientists the means to conduct additional clinical trials, publish findings and, ideally, find effective treatments for myriad conditions.

"We hope that California will sustain this, because stem cell research is the future of medicine," Anderson says. "And we're really just getting started."

Upcoming Clinical Trials

The California Institute for Regenerative Medicine (CIRM) Alpha Stem Cell Clinic (ASCC) Network was launched in 2015 to address a compelling unmet medical need for rigorous, FDA-regulated, stem cell-related clinical trials for patients with challenging, incurable diseases. Here are some of our clinical trials for which we are currently recruiting. For questions or inquiries about a clinical trial, contact stemcell@uci.edu or 949-824-3990. For a complete list of stem cell clinical trials, visit clinic.stemcell.uci.edu.

Phase 2b Trial to Evaluate Longeveron Mesenchymal Stem Cells to Treat Aging Frailty | ClinicalTrials.gov identifier: NCT03169231

Frailty in aging is estimated to affect over 10 percent of people ages 65 and older. Aging frailty results in increased fatigue, difficulty performing daily activities, decreased mobility, heightened risk of injury from falling, more frequent visits to the doctor and a general decrease in health lacking a definitive cause. This is a randomized, placebo-controlled, double-blind, parallel arm, multi-center Phase 2b study. The objectives of this study are to assess safety and efficacy of three doses of Longeveron Mesenchymal Stem Cells (LMSCs) compared to a placebo on: 1) functional mobility and exercise tolerance; 2) patient-reported physical function; and 3) the inflammatory cytokine biomarker tumor necrosis factor (TNF-a).

Autologous Dendritic Cells Loaded with Autologous Tumor-Associated Antigens for Treatment of Newly Diagnosed Glioblastoma | ClinicalTrials.gov identifier: NCT03400917

Glioblastoma (GBM) is the most common and aggressive form of malignant brain tumor. Median survival is only nine months, rising to 15–16 months for those who receive aggressive standard-of-care surgery and adjuvant chemoradiation. The cause of most GBM cases is unclear. The treatment developed by AIVITA is a platform technology applicable to any solid tumor type. It consists of autologous dendritic cells loaded with autologous tumor antigens from autologous self-renewing tumor-initiating cells, which means the cells have to be self-renewing as a cell line. This is a single-arm, open-label Phase 2 clinical trial in which approximately 55 patients will be enrolled with the intent to receive AV-GBM-1. Patients eligible for treatment will be those who 1) have recovered from surgery and are about to begin concurrent chemotherapy and radiation therapy (CT/RT); 2) for whom an autologous tumor cell line has been established; 3) have a KPS of > 70; and 4) have undergone successful leukapheresis, from which peripheral blood mononuclear cells (PBMC) were obtained that can be used to generate dendritic cells (DC).

Comparison of the Human Acellular Vessel (HAV) With Fistulas as Conduits for Hemodialysis | ClinicalTrials.gov Identifier: NCT03183245 | CIRM-funded

Nearly 500,000 Americans with kidney disease are on dialysis. The conduit for dialysis — called a human acellular vessel (HAV) — is implanted in the arm and used to carry the patient's blood to remove toxins. Current synthetic versions of this device have many problems, including clotting, infections and rejection. The main purpose of this study is to compare the HAV with arteriovenous fistula (AVF) when used for hemodialysis access. This is a Phase 3, prospective, multicenter, open-label, randomized, two-arm comparative study. Subjects who sign consent forms will undergo study-specific screening assessments within 45 days from the day of consent. Eligible study subjects will be randomized to receive either an HAV or AVF. The randomization will be stratified by upper arm or forearm placement based on the investigator's determination of where the study access (SA) should be located. Subjects will be followed to 24 months post SA creation at routine study visits regardless of patency status. After 24 months, AVF subjects with an unobstructed SA will be followed (while the SA remains open) for up to 5 years (60 months) post SA creation at routine study visits. After 24 months, HAV subjects will be followed (regardless of SA patency) for 5 years (60 months) post SA creation at routine study visits. This study has HYPERLINK RMAT designation from the FDA, meaning preliminary clinical evidence indicates that the device has the potential to meet an unmet medical need.

Stem Cell Community Lecture Series

Join our physician scientists and basic science researchers as they explore "hot topics" related to stem cells, clinical trials, therapies and the future of regenerative medicine. Community lectures are free and open to the public.

Low Oxygen & Bioengineered Vessel Repair
Tuesday, Dec. 11, 2018
Better Treatment for Kidney Disease?
Olga Razorenova, PhD
Roy Fujitani, MD

Rehab, Robots & Dance
Tuesday, Jan. 29, 2019
The Connection Between Physical Movement and Rehabilitation
Dave Reinkensmeyer, PhD
Kelli Sharp, DPT

Neuromuscular Disorders
Tuesday, Feb. 26, 2019
The Brainstorm Stem Cell Trial to Treat ALS
Tahseen Mozaffar, MD
Namita Goyal, MD

Bioengineering, Cell Sorting & Stroke
Tuesday, Mar. 26, 2019
Stem Cell Therapies for Stroke
Lisa Flanagan, PhD
Leonid Groysman, MD

Aging, Alzheimer's & Frailty
Tuesday, Apr. 30, 2019
Can We Find the Fountain of Youth?
Mathew Blurton-Jones, PhD
Lisa Gibbs, MD

Lectures are held at 7 p.m. in Thorp Conference Center, on the fourth floor of Gross Hall.
To register, visit stemcell.uci.edu/rsvp or call 949-824-2911.

Contact Us

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