

# USC Stem Cell NEWS

## USC hosts Calif. stem cell agency's 10th birthday bash

**F**ew 10 year olds have brought 10 potential medical treatments into clinical trials. The California Institute for Regenerative Medicine (CIRM), however, is a truly exceptional 10 year old.

In 2004, California voters created CIRM to dispense \$3 billion to fund stem cell research. This year alone, 10 CIRM-funded projects, including ones based at USC, are expected to be approved for clinical trials.

To celebrate this milestone, CIRM held a press day on November 20 at the Eli and Edythe Broad CIRM Center for Regenerative Medicine and Stem Cell Research at USC. This facility would not exist without CIRM funding, noted Carmen A. Puliafito, dean of the Keck School of Medicine of USC.

Jonathan Thomas, chairman of CIRM's governing board, called the first 10 years a "historical time in medical research."



Stem cells won the heart of clinical trial participant Fred Lesikar. (Photo by Cristy Lytal)

Cedars-Sinai's Dhruv Sareen added that "what Silicon Valley has done for technology, CIRM is doing for stem cell research in California."

ViaCyte's Eugene Brandon described the company's

clinical trials with an implantable stem cell device to treat type 1 diabetes.

City of Hope's John Zaia mentioned his collaboration with Sangamo BioSciences on a CIRM-funded HIV/

## About USC Stem Cell

USC Stem Cell is a collaborative and multidisciplinary effort working to translate the potential of stem cell research to the clinical imperative of regenerative medicine.

The initiative brings together more than 100 research and clinical faculty members from the Keck School of Medicine of USC, Children's Hospital Los Angeles, the USC Viterbi School of Engineering, the USC Davis School of Gerontology, the Ostrow School of Dentistry of USC, the USC School of Pharmacy, and the USC Dornsife College of Letters, Arts and Sciences. USC Stem Cell is also creating new educational opportunities with the USC Marshall School of Business and the USC Roski School of Art and Design.

AIDS clinical trial. CIRM also funded development of a genetic modification technique used in the trial and pioneered by Zaia, City of Hope's David DiGiusto, Sangamo scientists and USC's Paula Cannon.

Head of USC Stem Cell Andy McMahon underscored the importance of these and other clinical trials exploring treatments for Alzheimer's disease, macular degeneration, osteoarthritis and more.

UCLA's Gay Crooks reported how CIRM-supported clinical trials have already benefitted 18 children with "bubble baby disease." Fred Lesikar also shared his personal story of suffering a massive heart attack and enrolling in a stem cell-based clinical trial, which seems to have improved his health.

The last word was left to Bob Klein, who led the drive to create CIRM: "The genius of the people of this state is reflected in their commitment to this cause, and we should be grateful for their vision in supporting research that will save and transform people's lives."

# USC Stem Cell recruits nearly 20 new Ambassadors

In 2014, USC Stem Cell recruited nearly 20 Ambassadors for Stem Cell Research at USC. The Ambassadors hail from everywhere from San Diego to San Francisco, Hawaii to Beijing, and include leaders in medicine, venture capital, engineering, real estate, wealth management and philanthropy.

As advocates and supporters, these Ambassadors play a pivotal role in fulfilling the mission to revolutionize medicine through the discovery, translation and clinical application of stem cell research.

Through their support, USC Stem Cell has invested in interdisciplinary, disease-focused research initiatives,

promoted the careers of junior faculty and postdocs, established new facilities to find novel drugs, and provided inner-city youth with laboratory experience.

In November, USC Stem Cell's inaugural Ambassadors dinner featured special guest Bob Klein, author and architect of Proposition 71, the California Stem Cell Research and Cures ballot initiative. USC Stem Cell looks forward to hosting many more exciting Ambassador events in 2015.

For more information, contact Kelli-Ann Nakayama at [stemcelldev@usc.edu](mailto:stemcelldev@usc.edu) or (323) 442-1013.

## Postdoc Lori O'Brien receives first Broad Fellowship

What makes stem cells develop into kidneys? Lori O'Brien, a postdoctoral research associate in the laboratory of Andy McMahon, has received the first Broad Fellowship to help answer this question.

O'Brien is the first of a series of Broad Fellows, exceptional senior postdoctoral researchers at the transition point to starting their own stem cell laboratories. The fellowship was established as part of a \$2 million gift from The Eli and Edythe Broad Foundation to the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research at USC. The gift also supports core research facilities and innovative projects.

"One component of this money is to provide senior postdoctoral fellows with a year's worth of funding and their own lab support," said McMahon, director of USC's stem cell research center. "This enables our most promising young scientists to become the next generation of innovators in regenerative medicine."

The unanimous choice of the external review committee, O'Brien's project explores how key genes are "turned on" or "turned off" through a process called epigenetic regulation. Turning off specific genes in kidney stem cells prompts them to self-renew, or divide and give rise to more stem cells. These stem cells self-renew until they receive signals to specialize into

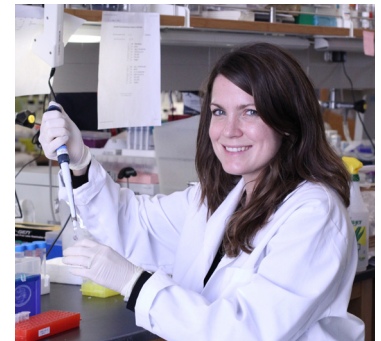
nephron cells, which form the functional unit of the kidneys.

O'Brien is focusing on the epigenetic regulator Phf19, which encourages embryonic stem cells to self-renew, and may have a similar effect on kidney stem cells.

O'Brien has always possessed a curious mind with a scientific bent. Born in Oshkosh, Wisconsin, she witnessed her first "dissection" when her dad, who liked to fish, would clean and filet his catch in the yard. She was fascinated by this early glimpse into fish anatomy — and all aspects of the natural world.

She parlayed her inquisitive mind into earning a bachelor's degree in bacteriology and a PhD in biochemistry from the University of Wisconsin-Madison. As a postdoctoral researcher, she has studied kidney development at Massachusetts General Hospital, Harvard University and USC.

"I'm honored to have been chosen by the committee," said O'Brien, "and thank The Eli and Edythe Broad Foundation for their generous contribution to establish the award."



Lori O'Brien  
(Photo by Cristy Lytal)

# General David H. Petraeus gets a four-star tour of USC

**D**avid H. Petraeus, retired four-star general and former CIA director, visited the Health Sciences Campus on Nov. 4 to see the view on the front lines of the life sciences revolution. Petraeus — who is also the Judge Widney Professor at



Graduate student Kimberley Babos with David H. Petraeus (Photo by Steve Cohn)

USC and Chairman of the KKR Global Institute — met with students and faculty across campus.

Head of USC Stem Cell Andy McMahon and screening director Justin Ichida welcomed Petraeus to the new Choi Family Therapeutic Screening Facility, which is testing drugs on motor neurons formed by reprogramming skin cells from patients with amyotrophic lateral sclerosis, or Lou Gehrig's disease.

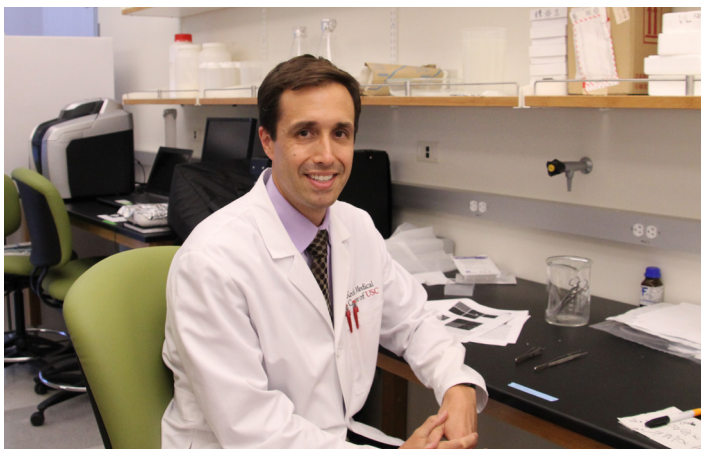
Professor of Research Neil Segil is collaborating with Ichida to apply a similar approach to hearing loss. His team has used cellular reprogramming to create inner ear cells, and plans to use these cells to search for drugs that protect against or reverse hearing damage.

Petraeus finished his day with medical school Dean Carmen A. Puliafito for an installment of the Dean's Distinguished Lecturer Series. "This has to be the Delta force of health science campuses," said Petraeus.

# Jon-Paul Pepper is a surgeon, scientist and student

**U**SC's Jon-Paul Pepper is not the average award winner. He's also not the average facial plastic surgeon, faculty researcher or master's student — in part, because he's currently all of these things.

Pepper has received the first Research Scholar Award from the Educational and Research Foundation for the American Academy of Facial Plastic and



Jon-Paul Pepper (Photo by Cristy Lytal)

Reconstructive Surgery (AAFPRS). The award provides two years of funding for his study on reprogramming skin-derived stem cells into nerve grafts for treating facial paralysis. He's tackling this project with Justin Ichida, assistant professor of stem cell biology and regenerative medicine.

Pepper — who joined USC's Department of Otolaryngology-Head and Neck Surgery as an assistant professor in Fall 2013 — specializes in the reconstructive surgery of the face. He believes that stem cells are the future of facial nerve reanimation, and is enrolled in USC's new master of science in stem cell biology and regenerative medicine program.

Pepper is also collaborating with USC Stem Cell principal investigator Mark Humayun on a clinical trial that explores the electrical stimulation of facial nerves as a treatment for Bell's palsy, a condition that causes sudden and unexplained facial paralysis.

"I have an interest in not only developing my clinical practice but also performing impactful research," said Pepper. "So I was ecstatic to be recruited to USC."



# Research Highlights

**Yi Liu, Songtao Shi and colleagues** demonstrated that transplanting stem cells from human baby teeth into mice ameliorates osteoporosis. They found evidence suggesting that these human stem cells modulate the mice's immune systems, rescuing bone marrow stem cells and promoting bone growth. (*Journal of Dental Research*)

**David Hinton's group** arranged the specialized cells that support the health of the retina — known as retinal pigment epithelial (RPE) cells — in a single polarized layer on a membrane. They showed that this organized layer of cells survives much better than isolated cells in an environment typical of the eyes of patients with age-related macular degeneration (AMD). This supports the strategy that Hinton and his colleague Mark Humayun will be using in an upcoming phase 1 clinical trial for patients with the dry form of AMD. (*Stem Cells Translational Medicine*).

**Yvonne Leung, Eve Kandyba, Krzysztof Kobiela and colleagues** discovered a new population of nail stem cells, which have the ability to either self-renew or undergo specialization or differentiation into multiple tissues. The researchers are now wondering whether or not the right signals or environmental cues could induce these nail stem cells to generate additional types of tissue — potentially aiding in the repair of everything from nail and finger defects to severe skin injuries and amputations. (*Proceedings of the National Academy of Sciences*)

**Akio Kobayashi, currently at the University of Washington, worked with Andy McMahon and his lab** to identify a progenitor cell population that is

present during kidney formation. This cell population expresses a unique protein called Foxd1, and gives rise to all other kidney cell types surrounding the filtering unit, known as the nephron. (*Stem Cell Reports*)

**Alan Wayne and collaborators at the National Cancer Institute** published the results of a phase 1 clinical trial using white blood (T) cells that have been genetically engineered to target cancer cells and treat acute B lymphoblastic leukemia. The study revealed that engineered white blood cells are safe and show anti-cancer activity in children and young adults. (*The Lancet*)

## Featured Image



In fall 2014, USC welcomed its inaugural class of more than 30 master's students in stem cell biology and regenerative medicine to one of the first programs of its kind in the United States. (*Photo by Cristy Lytal*)

## Your Support

To learn how you can transform medicine by supporting USC Stem Cell, contact:

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