SUE & BILL GROSS
STEM CELL RESEARCH CENTER
UNIVERSITY of CALIFORNIA · IRVINE

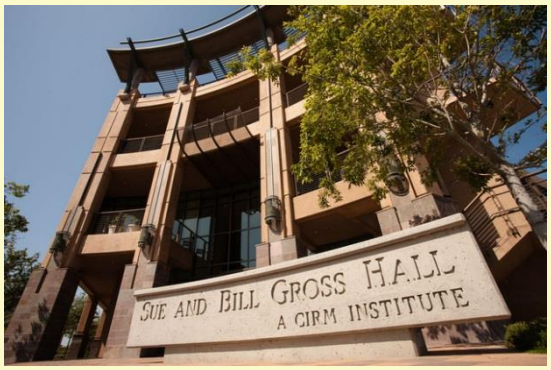
This is a very exciting issue as we have quite a bit of news to share with you - research updates, distinguished awards, grants received, **new tour dates** and photos from our 10th Anniversary Celebration.

We hope you enjoy this issue. If there are topics you would like to hear more about, please feel free to email me with your suggestions and requests.

Until next time,

Janice Briggs

jbriggs@uci.edu



Sidney H. Golub, PhD
Director, Sue & Bill Gross
Stem Cell Research Center

From the Director's Desk

Bright Past, Brilliant Future

August, 2016

A decade is an interesting unit of time. While just a fraction of a lifetime, a decade is long enough that we can see broad social and cultural changes. In the world of medical science, the past 10 years have been a period of momentous change, and scientists, journalists and policy makers have referred to this period as the Stem Cell Decade. It was in 2006 that California started awarding stem cell research grants through its publicly supported Prop 71 initiative that created the California Institute for Regenerative Medicine (CIRM). That year also marked the scientific development of a new kind of stem cell, the "induced pluripotent stem cell" or iPS

cell. This discovery, which won the Nobel Prize in 2012, allowed scientists to create stem cells very similar to those found in embryos by the genetic reprogramming of normal adult cells. This opened up many new possibilities in stem cell research.

2006 was also an important year at UCI as it was in 2006 that the Sue & Bill Gross Stem Cell Research Center was founded. We recently marked the 10th anniversary of our creation and were joined at that celebration by many of our founding faculty. It was an opportunity to reflect on this exciting era as we reviewed our many accomplishments in research, education, and the bringing of stem cells to human clinical care.

We used this opportunity to thank our founding supporters who made this center possible. We expressed our great appreciation to our lead donors Sue & Bill Gross and our other founding donors the Thorp family, Maggie & Ed Chang, and Tom & Misako Yuen for their vision in launching this enterprise. Vice-Chancellor for Health Howard Federoff presented the Grosses with a gift of a lovely picture of stem cell derived nerve cells as a memento of the event. We also thanked our more recent supporters, who have enabled our new seed grant program to build on our faculty's best new ideas. It was a wonderful occasion to express our continuing gratitude to our supporters.

While it is gratifying to look back on what we have accomplished at the Stem Cell Research Center, it is very exciting to look at our current accomplishments. A few examples of the reasons we are excited:

- Maksim Plikus was given the prestigious recognition and research support as one of 22 Pew Scholars in the USA appointed in 2016. Max was recognized for his outstanding work on stem cells in wound healing.
- The UCLA-UCI Alpha Stem Cell Clinic has been approved for new clinical studies at UCI of stem cells in the treatment of stroke, the brain

tumor gliobalstoma, traumatic brain injury, and Alzheimer's disease.

- Henry Klassen will be featured in the September issue of National Geographic with its cover story about fighting blindness.
- Jonathan Lakey was awarded a major new grant from the JDRF (Juvenile Diabetes Research Foundation) for his work on islet cell transplantation.
- Leslie Thompson and her team studying Huntington's Disease won a coveted NIH Program Project grant to advance their work on the nature and causes of this disease.

These and other exciting success show that the path we started on a decade ago continues in an exciting and promising direction. Thank you for your support and I hope you will visit us at the Stem Cell Research Center and see for yourself why we are so excited.

RESEARCH NEWS

Stem cell therapy for retinitis pigmentosa found safe, well-tolerated in first-stage clinical trial

Sue & Bill Gross Stem Cell Research Center researchers have reported that a therapy they created to cure retinitis pigmentosa is safe and well-tolerated, with no immunological issues, by the first group of patients enrolled in an ongoing phase I/II clinical trial. Led by the regenerative medicine company jCyte, the trial has successfully undergone four reviews by the Food & Drug Administration's data and safety monitoring board. The first participant in the

study, which involved injecting human retinal progenitor cells into one eye, has now been followed clinically for one year post-treatment. Six- to 12-month safety results from this patient and the eight that followed are encouraging, said Dr. Henry Klassen, an associate professor of ophthalmology at UCI and a jCyte co-founder. The cell-based approach is intended to rescue sick and dying retinal photoreceptor cells (rods, cones) in a diseased retina. The trial is being conducted at UCI's Gavin Herbert Eye Institute and at Retina-Vitreous Associates in Los Angeles. Further clinical studies are being planned.

With JDRF grant, UCI professor pursues approach to diabetes that makes daily injections obsolete

Unlike Type 2 diabetes, Type 1 diabetes often afflicts the young with an indifference to lifestyle and dietary habits. It begins when the immune system attacks the insulin-producing cells in the pancreas. Insulin is necessary for balancing blood sugar levels, and it allows the body to use these sugars for energy or store glucose for future use. Without this hormone, blood sugar levels will skyrocket, leading to severe health problems and even death.

Type 1 diabetes – also known as juvenile diabetes – is considered a chronic disease that must be managed with insulin injections – every day, for the rest of a person's life.

Jonathan Lakey, a University of California, Irvine professor of surgery and biomedical engineering, is a pioneer in an emerging approach to treating Type 1 diabetes, an approach that frees patients from the daily needle jabs. In 2000, he helped create the Edmonton Protocol, a surgical method by which cell clusters called islets can be transplanted into the pancreas to stimulate insulin production, making injections unnecessary. It's been shown to work, but there are still many hurdles to overcome, such as immune system rejection of the transplanted cells.

With a new $1.3 million grant from the JDRF (formerly the Juvenile Diabetes Research

Foundation), Lakey, UCI associate professor of surgery and biomedical engineering Elliot Botvinick and Paul de Vos of the University of Groningen in the Netherlands are continuing to seek a way to transplant insulin-secreting, encapsulated, stem cell-derived islet cells that the immune system will not attack.

read more Needles not needed

UCI study finds safer stem cell-derived therapy for brain radiation recovery

Microvesicle injections restored cognition without adverse side effects

While stem cells have shown promise for treating brain regions damaged by cancer radiation treatments, University of California, Irvine researchers have found that microscopic vesicles isolated from these cells provide similar benefits without some of the risks associated with stem cells.

In research with rats, these membrane structures secreted by cells – called microvesicles – transplanted two days after cranial irradiation restored cognitive function, reduced inflammation and protected neurons, as measured in four- and six-week assessments, with no sign of immunorejection or tumor growth – two stem cell-related risk factors. Study results appear this week in the early online edition of *Proceedings of the National Academies of Science*.

Microvesicles are small, fluid-filled sacs secreted by all human cells. Their plasma contains a range of bioactive cargo (proteins, RNAs, etc.) that can benefit cellular physiology. In the brain, they help regulate the health and functionality of neurons. Microvesicles also can play an important role in tissue regeneration. safer stem cell-derived therapy

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Distinguished Awards

Academic Senate's Distinguished Faculty Awards for 2016-2017

Congratulations to **Sidney Golub, PhD** and **David Reinkensmeyer, PhD** recipients of the Academic Senate's highest honors. The awards are given to Senate members who have achieved excellence through their activities in research, teaching and service. The Academic Senate's Distinguished Faculty Awards are selected by the Committee on Scholarly Honors and Awards. Please join me in congratulating our colleagues on their outstanding achievements.

Daniel G. Aldrich, Jr. Distinguished University Service Award

Professor Emeritus Sidney Golub

Department of Microbiology and Molecular Genetics

Distinguished Mid-Career Faculty Award for Research

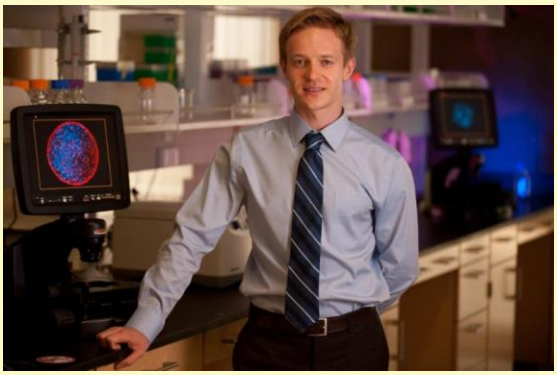
Professor David Reinkensmeyer

Department of Anatomy and Neurobiology

Developmental biologist Maksim Plikus named 2016 Pew Scholar

Maksim Plikus, assistant professor of developmental & cell biology, is among the exceptional early-career scientists named a 2016 Pew Scholar in the Biomedical Sciences. For more than 30 years, the Pew Charitable Trusts have supported outstanding biomedical researchers at the start of their careers, and this year's class of 22 scholars is drawn from prestigious institutes across the country. Each scholar will receive flexible funding for four years to pursue foundational, innovative research. Pew will award Plikus $240,000 to support his work on how wounds in the skin can heal without scarring. His research aims to show that under certain circumstances the stem cells in scar tissue can be reprogrammed into new, useful cell types that encourage fresh skin growth. "The regeneration mechanism we will study with the help of Pew funding is likely universal to scarring in organs other than skin," said Plikus, who is a member of the Sue & Bill Gross Stem Cell Research Center. "We envision that pharmacological augmentation of this mechanism could have a transformative effect on the anti-scarring therapies, which are currently minimally effective." Plikus earned a doctorate in pathology at USC and joined the UCI faculty in 2012.

Developmental biologist Maksim Plikus studies how stem cells can be reprogrammed to heal wounds and avoid skin scarring. Daniel A. Anderson / UCI



Edward A. Dickson Emeritus Professor for 2016-2017

Sidney Golub, PhD was appointed as the Edward A. Dickson Emeritus Professor for 2016-2017. This is a nice honor which is afforded to one or two emeritus faculty at each campus each year. It comes with a budget of $10,000 which I will use to expand our bioethics activities.

Programs

The UCI Training Program in Stem Cell Translational Medicine for Neurological Disorders

The UCI Sue & Bill Gross Stem Cell is the home to a unique training program for young scientists directed by Profs. Leslie Thompson and Peter Donovan.

For the promise of stem cells to be more broadly realized, we need advances in the basic research in this rapidly evolving field and these advances must be translated through pre-clinical and clinical development into clinical practice. Under the leadership of Profs. Leslie Thompson and Peter Donovan, we have devised a training program that prepares young scientists in the clinical aspects of the disease they are studying, the challenges of preclinical safety and efficacy testing, the complexities of the regulatory processes required for human clinical trials, and the business aspects of taking research products to patients. The goal of this NIH funded Training

Program in Stem Cell Translational Medicine for Neurological Disorders is to train a new generation of scientists in the translational application of stem cell biology to neuroscience. Especially important will be the combination of real and unique engagement with clinical faculty and the opportunity of internships with successful pharmaceutical and biotech companies. One of our trainees, Milad Riazifar (pictured), who is working on stem cell biology in the laboratory of Dr. Weian Zhao, will be among our first industry interns and has started at the industry leading pharmaceutical and biotechnology company Amgen, Inc for a three month internship.

Padre Foundation makes $25,000 gift to support Juvenile Diabetes Research

We are pleased to announce that the PADRE Foundation is supporting Dr. Jonathan Lakey's diabetes research with a $25,000 gift. Since 1985, the Pediatric-Adolescent Diabetes Research and Education (PADRE) Foundation has been dedicated to improving the lives of the youth and families living with Type 1 Diabetes by providing exceptional education, support and events for all

Thanks to our Donors!

Special thanks to our donors for gifts of $10,000 and above received since July 1, 2015:

Anne Brownstein

Annie and Clement Chu, MD

Mary & Emmanuel Foundation

Mike Johnson Family

M.N. Mansour, PhD

Selma McNally Estate

Marcia C. Mills, PhD

Padre Foundation

Char and Sam Salkin

Susan Scott Foundation

Edward O. Thorp, PhD

children with diabetes, as well as for their parents and siblings. PADRE seeks to inspire kids to lead amazing lives until a cure is found. PADRE was founded by a group of parents who recognized the need in Orange County for education, resources and support for families who have children with diabetes. Today, PADRE serves more than 2,500 pediatric patients throughout Southern California. PADRE also supports diabetes research, so that detection, prevention and management of diabetes can be improved and ultimately, to help towards a cure. We are proud to have the PADRE Foundation as a partner.

UPCOMING TOURS


SUE & BILL GROSS
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Tour Sue & Bill Gross Stem Cell Research Center

Fascinated by cutting-edge science? Ever wondered about the discoveries being made at UC Irvine?

Now you can get a behind-the-scenes peek and learn first hand about the hope that stem cells offer.

Tour Date:

Wednesday, September 14, 2016–10am-11:30am

Guest Speakers:
Daniela Bota, MD, PhD
Associate Professor of Neurology
Medical Director of Neuro-Oncology Program
Dr. Bota's topic will focus on stem cells for "Chemo Brain"

Michael Zaragoza, MD, PhD
Assistant Clinical Professor
Dr. Zaragoza's topic will focus modeling the heart with stem cells.

Daniela Bota, MD, PhD

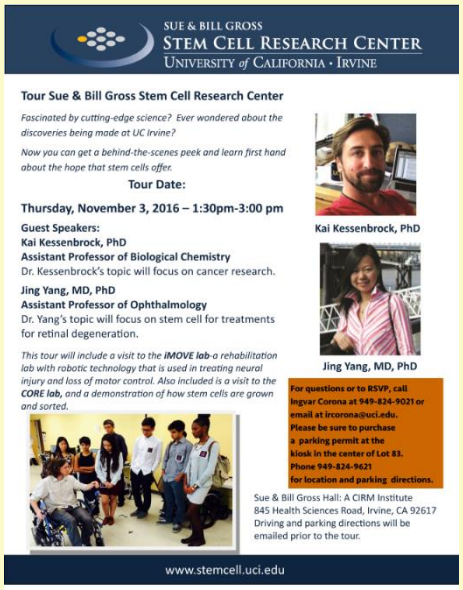
Michael Zaragoza, MD, PhD

For questions or to RSVP, call Ingvar Corona at 949-824-9021 or email at ircorona@uci.edu. Please be sure to purchase a parking permit at the kiosk in the center of Lot 83. Phone 949-824-9621 for location and parking directions.

Sue & Bill Gross Hall: A CIRM Institute
845 Health Sciences Road, Irvine, CA 92617
Driving and parking directions will be emailed prior to the tour.

www.stemcell.uci.edu

September 14, 2016 10:00 -11:30am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Tour Date:

Thursday, November 3, 2016 – 1:30pm-3:00 pm

Guest Speakers:
Kai Kessenbrock, PhD
Assistant Professor of Biological Chemistry
Dr. Kessenbrock's topic will focus on cancer research.

Jing Yang, MD, PhD
Assistant Professor of Ophthalmology
Dr. Yang's topic will focus on stem cell for treatments for retinal degeneration.

*This tour will include a visit to the **iMOVE lab**-a rehabilitation lab with robotic technology that is used in treating neural injury and loss of motor control. Also included is a visit to the **CORE lab**, and a demonstration of how stem cells are grown and sorted.*

Kai Kessenbrock, PhD

Jing Yang, MD, PhD

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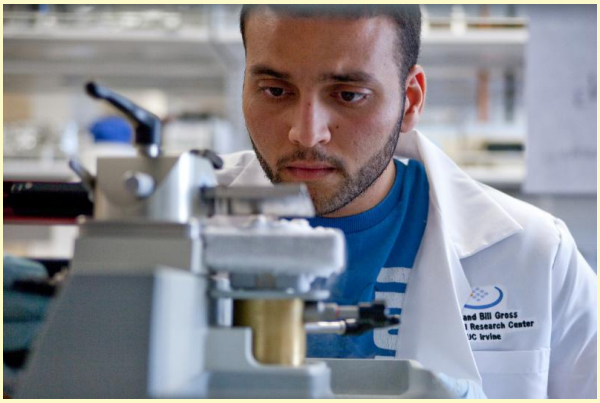
November 3, 2016 1:30-3:00pm

January 25, 2017 10:00- 11:30am

From stroke to Alzheimer's disease to multiple sclerosis, scientists are closer than ever to understanding stem cells and how they might halt, even reverse the effects of degenerative conditions.

Your gift, no matter how large or small, supports our scientists as they push the boundaries of what is possible.

Please consider Sue & Bill Gross Stem Cell Research Center in your year end giving.



DONATE NOW

The IRA Charitable Rollover

If you are aged 70 ½ or over, you can once again make tax-advantaged gifts to the Sue & Bill Gross Stem Cell Research Center from your traditional IRA.

Through the IRA charitable rollover, you also help yourself. The amount that you transfer to the UCI Foundation to benefit the stem cell research center qualifies as your required minimum distribution, **and** it will not get counted in your taxable income, potentially saving you in taxes while preserving benefits you may be receiving through the state.

Learn more about the charitable IRA here.

Or Contact Roland Ho at roland.ho@uci.edu 949-824-6454.

EVENTS

10th Anniversary Celebration
April 19, 2016

Dr. Federoff, Sue Gross and Bill Gross

L to R: Hans Keirstead, PhD, Sue Gross, Bill Gross, Ed Thorp, PhD, Catherine Baldwin, Howard Federoff, MD, PhD.

Howard Federoff, MD, PhD, Sue Gross and Bill Gross

L to R: Ellen Carmack, Jonathan Lakey, PhD, Anne Brownstein

Dr. Geneva Matlock and friend

L to R: Peter Donovan, PhD, Maggie Chang, Ed Chang

Bill and Sue Gross, Dr. Sidney Golub