

PRESS RELEASE

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## **SIX UCLA SCIENTISTS AWARDED \$8 MILLION IN BASIC BIOLOGY GRANTS FROM THE STATE STEM CELL AGENCY**

Six scientists with the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA were awarded more than \$8 million in grants from the state stem cell agency on May 3 to investigate basic mechanisms underlying stem cell biology and differentiation.

The Basic Biology III grants – 27 awarded in all totaling \$37.7 million – represent an on-going effort by the California Institute of Regenerative Medicine (CIRM) to build a foundation to support future research designed to take stem cell science from the laboratory bench into the clinic. The studies supported by these awards will form the foundation for future translational and clinical advances, enabling the realization of the full potential of human stem cells and reprogrammed induced pluripotent stem cells for therapies and as tools for biomedical innovation.

To date, UCLA and its stem cell scientists have received 49 grants from CIRM totaling more than \$148.6 million.

Dr. Owen Witte, director of the Broad Stem Cell Research Center, said the recognition from CIRM for the “breadth and very high quality of the basic science at UCLA’s stem cell center is very rewarding.”

“The science supported by these grants will propel future translational and clinical advances in areas such as cardiovascular disease and blood and neurological disorders, and hopefully will result in new, more effective therapies for people who suffer from these diseases,” Witte said.

Broad Stem Cell Research Center scientists receiving the grants include Dr. Gay Crooks, a professor of pediatrics and pathology and laboratory medicine (\$1,375,983); Shuo Lin, a professor of molecular, cell and developmental biology in Life Sciences (\$1,382,400); William Lowry, an assistant professor of molecular, cell and developmental biology in Life Sciences (\$1,354,230); Dr. Robb MacLellan, an associate professor of cardiology (\$1,181,306); Kathrin Plath, an assistant professor of biological chemistry (\$1,364,598); and Yi Sun, an associate professor of psychiatry and biobehavioral sciences and molecular and medical pharmacology (\$1,382,400).

Crooks’ grant-funded project seeks to answer fundamental questions about the mechanisms by which pluripotent stem cells differentiate into mature tissue. She and her team are hoping to discover if the cell types produced during differentiation of the pluripotent stem cells produce the microenvironment needed for specialized tissue stem cells to develop. These discoveries could help realize the clinical potential of pluripotent stem cells for use in regenerative medicine by making the process by which tissues are generated from these cells more efficient and controlled.

Lin's grant will fund efforts to develop a protocol for the differentiation of human embryonic stem cells into vascular endothelial cells for the study and treatment of cardiovascular disease, including heart disease, heart failure and stroke. Cardiovascular disease is caused by damage to blood vessels, and the ability to repair this damage will improve outcomes. Lin and his team have identified a critical gene in the development of blood vessel precursor cells and propose to use it to optimize the generation of blood vessel cells from human embryonic stem cells.

Lowry's project seeks to determine whether manipulating expression of certain genes in pluripotent stem cells can bring the cells closer in their properties to mature tissue cells, making them functional equivalents to the adult stem cells lost in disease or injury. Lowry and his team also propose to discover small molecule compounds that can have the same effect as the genetic manipulation. Such compounds could allow for appropriate maturation of all types of pluripotent stem cell derivatives, resulting in successful transplantation of these cells for the treatment of a variety of diseases and injuries.

MacLellan's project hopes to address deficiencies in the understanding of the cardiac niche, a microenvironment about which very little is now known. MacLellan and his team hope to uncover the niche's effects on the expansion and differentiation of cardiac progenitor cells, which are capable of differentiating into all three cell types of the heart and could potentially be used to repair heart damage. They also hope to utilize novel bioengineering approaches to fabricate synthetic niche environments, both for studying the role of the niche in cardiac progenitor cell biology and to aid in the advancement of regenerative medicine.

Plath's grant will fund studies into the regulation of X chromosome inactivation in human cells using human induced pluripotent stem cells, or cells reprogrammed to have all the abilities of human embryonic stem cells, as a model system. A normal female has two X chromosomes and must shut off one of two during embryonic development by inducing inactivation. Plath believes her studies will unveil mechanisms underlying this fundamental silencing process and human development that may be instrumental for the careful characterization of human pluripotent stem cells states.

Sun's project seeks to develop a new way to monitor the connectivity and function of transplanted neurons *in vivo*. Sun and her team will engineer two types of neurons from normal human embryonic stem cells and an human embryonic stem cell model of Rett Syndrome, an autism spectrum disorder, that can be modulated by a light dependent switch. They will study the fate of these cells in a small animal model to study the neurotransmission/connectivity characteristics of normal and diseased human neurons.

The stem cell center was launched in 2005 with a UCLA commitment of \$20 million over five years. A \$20 million gift from the Eli and Edythe Broad Foundation in 2007 resulted in the renaming of the center. With more than 200 members, the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research is committed to a multi-disciplinary, integrated collaboration of scientific, academic and medical disciplines for the purpose of understanding adult and human embryonic stem cells. The center supports innovation, excellence and the highest ethical standards focused on stem cell research with the intent of facilitating basic scientific inquiry directed towards future clinical applications to treat disease. The center is a collaboration of the David Geffen School of Medicine, UCLA's Jonsson Cancer Center, the Henry Samueli School of Engineering and Applied Science and the UCLA College of Letters and Science. To learn more about the center, visit our web site at <http://www.stemcell.ucla.edu>.