CIRM Awards Three Awards to UCSD Researchers in Support of Innovative Technologies

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The California Institute for Regenerative Medicine (CIRM) has awarded three grants totaling nearly $5.8 million to researchers at the University of California, San Diego for development of innovative technologies designed to advance translational stem cell research. The grants are part of $32 million in Tools and Technology Awards II awarded to 19 projects at 10 California institutions that were announced by CIRM today.

The CIRM Tools and Technology Awards II support the inception, early stage development and evaluation for stem cell research applications of innovative tools and technologies that will overcome current roadblocks in translational stem cell research.

"Translational" research refers to work targeted to convert basic research findings into actual treatments, diagnostics, or therapies for people; these awards are to help accelerate the practical applications of discoveries made in the laboratory.

A $1.8 million grant was awarded to Larry Goldstein, PhD, professor, Howard Hughes Medical Investigator and director of the UCSD Stem Cell Program, and Gene Yeo, PhD, assistant professor and member of the Institute for Genomic Medicine, both of the Department of Cellular & Molecular Medicine. Their translational research project aims to develop a cost-effective, non-labor intensive method of generating and characterizing human induced pluripotent stem cells (hIPSCs.) Their proposal combines new technology and scaling methods that will enable routine generation of tens to hundreds of independent hIPSC lines from human patients.

"Many diseases, including Alzheimer's disease, are thought to have a genetic basis. Identifying how this genetic variation contributes to disease susceptibility and drug response will require hIPSC lines from many patients," said Goldstein, adding that currently such hIPSC generation is bottlenecked due to the high cost and labor intensive nature of such work. "If successful, our project will lead to breakthroughs in understanding disease and more cost-effective development of better therapies."

The second grant, for just under $2 million, was awarded to Karl Willert, PhD, assistant professor of Cellular & Molecular Medicine and Director of the Human Embryonic Stem Cell Core Facility at UC San Diego, for his translational project to generate a panel of cell lines carrying gene-specific modifications.

Possibly the most exciting application of human embryonic stem cells (hESCs) in the clinic is in the area of regenerative medicine, where hESC-derived cells could be used to replace damaged cells or tissues. However, a major safety concern when hESC-based replacement therapies has been the potential risk of tumor growth.

Using a novel technology to introduce genes in hESCs, Willert proposes to develop a technology that will identify hESC cells of interest to researchers while eliminating undesirable or contaminated cell lines.

"Our hope is to develop a critical new tool that can help overcome safety concerns of tumor growth associated with embryonic stem cell replacement therapies," said Willert. "In addition to advancing the field of regenerative

medicine, such technology will be of immense value for scientists who study the intricate development processes of various cell types."

He added that the technology will also benefit scientists studying the roles of particular genes, and enable them to engineer specific mutations into genes associated with human diseases.

The third grant, also for just under $2 million, was awarded to University Professor Shu Chien, MD, PhD, in the Department of Bioengineering at the Jacobs School of Engineering; and co-investigators Karl Willert and Shyni Varghese, assistant professor of bioengineering.

Their translational research proposal is to develop synthetic microenvironments for stem cell growth and differentiation. Due to the high cost and limited range of testing capability, previous studies on factors affecting stem cell growth have focused on only one or a few elements of the cellular microenvironment. In addition, most cultures use non-human products in which to grow human pluripotent stem cells (hPSCs), which increases the potential for complications.

By developing a high-throughput cellular microarray screening tool that incorporates synthetic, rather than animal-based, materials, the researchers hope to identify the optimal conditions to support self-renewal and differentiation of hPSCs.

"This cost-effective technology - which will be made freely available to the biomedical community - will allow scientists to screen the effect of thousands of different conditions on the growth and maintenance of human pluripotent stem cells in the lab, allowing us to optimize culture conditions without exposing these cells to animal-derived products," said Chien, who also directs the UC San Diego Institute of Engineering in Medicine.

The grants boost total CIRM funding for UCSD projects to nearly $83 million since the first awards were given in 2006.

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