

CIRM Awards \$5.8 Million to Two UCSD Researchers for Early Translational Research Studies

Awards boost CIRM funding to UC San Diego to more than \$78 million

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Two scientists at the University of California, San Diego School of Medicine - Catriona Jamieson, MD, PhD, and Alysson Muotri, PhD - have received grants from the California Institute for Regenerative Medicine (CIRM) for stem cell research. The grants are part of \$67 million in Early Translation II Awards announced by CIRM today, which are designed to move research out of the lab and into the clinic.

Jamieson, assistant professor of medicine and director of stem cell research at UCSD's Moores Cancer Center, received a grant for \$3,341,758 to develop a panBCL2 antagonist as a dormant leukemia stem cell survival inhibitor. While also common in adults, leukemia is the most frequent form of cancer in children and teenagers. Chemotherapy has vastly improved the outcome for patients with leukemia, but many still die due to recurrence and development of drug-resistance in leukemia cells.

"Current leukemia approaches fail to eradicate the stem cells that can remain in a 'quiescent phase' after initial treatment," said Jamieson. After evading cell death during chemotherapy treatment, the persisting stem cells can give rise to leukemia clones that then begin to proliferate, resulting in recurrence of the disease and development of drug-resistance in leukemia cells. "Our approach aims to improve current leukemia therapy, with the hope of reducing a patient's risk of leukemia relapse and possible reduction of what are currently life-long drug treatments for the disease."

The proposed approach looks to eliminate leukemia stem cells by targeting BCL2 family members and tyrosine kinases that fuel survival, establishing therapeutic efficacy in novel cancer stem cell assays. In collaboration with Coronado Biosciences, the researchers will then develop methods for large-scale production, analysis and toxicity studies to advance the panBCL2 inhibitor to pre-clinical and clinical development.

The CIRM board also awarded a \$1,481,471 million grant to Alysson Muotri, PhD, assistant professor of pediatrics, to help design a future drug-screening system for autism spectrum disorders (ASD) that employs human neurons. Muotri and colleagues are using induced pluripotent stem cells derived from skin cells taken from patients with autism spectrum disorders like Rett Syndrome to create "autistic" neurons. The researchers believe this first-ever human cell model of ASD will provide a novel effective and insightful tool for diagnosing and developing personalized treatments of autistic syndromes. The CIRM grant supports initial studies of how specific pharmacological compounds interact with ASD human neurons.

"These are experiments that have never been possible before," said Muotri. "We hope they will help us better understand the causal molecular mechanisms of ASD, find possible biomarkers for the disease and identify specific therapeutic targets."

Autism affects one in 110 children in the United States, according to the Centers for Disease Control and Prevention. An estimated 1.5 million Americans live with the effects of the disease, which occurs in all ethnic and

socioeconomic groups and affects every age group, though males are four times more likely to have ASD than females.

The grants to Jamieson and Muotri boost total CIRM funding for UCSD projects to more than \$78 million since the first awards were given in 2006.

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