CIRM, the UK and Canada Award More Than $250 Million to Researchers to Accelerate Pace of Bringing Stem Cell Therapies to the Clinic

$20 million awarded to Moores UCSD Cancer Center Team

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Debra Kain

Researchers led by Moores UCSD Cancer Center Director Dennis A. Carson, MD, professor of medicine, and Catriona Jamieson, MD, PhD, assistant professor of medicine and director of the Cancer Stem Cell Research Program at the Moores UCSD Cancer Center have been awarded $20 million over four years to develop novel drugs against leukemia stem cells.

CIRM's 29-member Governing Board voted to approve funding to 14 multidisciplinary teams of California researchers. The four-year grants mark the first CIRM funding explicitly expected to result in FDA approval for a clinical trial. The Disease Team Research Awards fund research teams that include basic scientists, clinicians and industry. These collaborations speed the process of establishing clinical trials by avoiding mistakes being discovered late in the process and insuring that clinically relevant issues are considered early.

"This award will fund a team - including researchers from disparate disciplines and key industry-academic partners - to develop novel therapies targeting leukemia stem cells, with the goal of moving to clinical trials in the shortest possible time frame," said Jamieson, who was involved in a unique partnership between industry and academia that, in 2008, led to human clinical trials of a new drug for a rare class of blood diseases in just one year's time. "Throughout California, scientists and physicians working in stem cell research are keeping their eyes on the goal of getting these promising therapies to patients as quickly, and safely, as possible."

More than in any other cancer, scientists understand the molecular changes in the blood-forming cells that cause leukemia, but it has been difficult translating scientific results into new and effective therapies. Despite current drug therapies, small numbers of leukemia stem cells remain in patients, and continue to grow, spread and kill normal cells. Experimental results suggest that it will be possible to destroy leukemia stem cells using drugs or drug combinations with minimal damage to normal cells. The research team will develop six existing candidate molecules targeting leukemia stem cells - but not normal, blood-forming or hematopoietic stem cells - and these will be tested against both chronic and acute forms of leukemia.

The Moores UCSD Cancer Center group will collaborate with a Canadian research team led by pioneering leukemia stem cell scientist John Dick, PhD, at the University of Toronto.

"We are gratified to learn that CIRM once again has recognized the ingenuity, commitment and efforts of stem cell scientists at UC San Diego Health Sciences and provided them such generous support," said David Brenner, MD, Vice Chancellor of Health Sciences and dean of the UC San Diego School of Medicine.

Each disease team will be actively managed by CIRM and the agency's international partners for those teams with cross-border collaborations. They will receive incremental funding upon achieving set milestones in the path to the clinic.

Several additional UC San Diego School of Medicine researchers are part of disease teams funded to develop novel stem-cell therapies to fight amyotrophic lateral sclerosis (ALS or Lou Gehrig's disease) and brain tumors.

Larry Goldstein, PhD, professor of cellular and molecular medicine at the UC San Diego School of Medicine, Howard Hughes Medical Institute Investigator and director of the UC San Diego Stem Cell Program, and Don W. Cleveland, PhD, professor and chair of cellular and molecular medicine at the UC San Diego School of Medicine and head of the Laboratory of Cell Biology at the Ludwig Institute for Cancer Research (LICR), are co-principal investigators on a $10.8 million grant to Samuel Pfaff at the Salk Institute to develop a novel cellular therapy for ALS.

Pfaff, Professor and Helen McLoraine Developmental Chair in Neurobiology at the Salk Gene Expression Laboratory, focuses on investigating how motor neurons form and make their predetermined connections between the spinal cord and the muscles in the body.

ALS is a devastating disease marked by motor neuron degeneration, leading to extreme muscle weakness, paralysis and death. Previous research supports the transplant of glial (cells surrounding the motor neurons) as a possible treatment for ALS, and studies of precursor cells that develop into a type of glial cell called astrocytes have shown promise in animal studies. The researchers hope to develop an ALS therapy based on using human embryonic stem cells to create astrocyte precursor cells for transplant into patients, where the hope is the astrocytes would mature into new and healthy glial cells.

Webster Cavenee, PhD, director LICR and professor of medicine at UC San Diego School of Medicine, is co-principal investigator of a $19.2 million grant to UC San Francisco for a six-institution study of the effectiveness of using neural stem cell-based gene therapy to fight brain tumors. The disease team, led by principal investigator Mitchel Berger of UCSF, also includes researchers from UCLA, the Salk Research Institute, LICR, and the Burnham Institute.

Brain tumors are incurable, and current therapies are limited by the inability to reach widely spread tumor cells throughout the brain. But neural stem cells have an affinity for tumor cells, and the researchers are planning to attach payloads of "killer genes" to the stem cells, hoping that the stem cells will be drawn to the tumors, killing them. The team will test the effectiveness of human neural stem cells and two different types of genes in various mouse models of a type of deadly brain tumor, glioblastoma.

The Disease Team Research Awards include approximately $8 million from the Medical Research Council, UK, and $35 million from the Cancer Stem Cell Consortium, Canada, to fund the international portions of the collaborations.

CIRM President Alan Trounson said the pace of the Disease Team projects stands in contrast to the decade or more that's usually required to reach clinical trials. "Scientists have talked for years about the need to find ways to speed the pace of discovery. By encouraging applicants to form teams composed of the best researchers from around the world we think CIRM will set a new standard for how translational research should be funded," he said.

Other diseases being targeted by the teams include HIV/AIDS, type1 diabetes, macular degeneration, heart attack and stroke, sickle cell anemia and skin disease.

The disease team grant to UC San Diego brings its total CIRM funding to nearly $65.6 million since the first funding was awarded in 2006.

Media Contacts: Debra Kain, 619-543-6163, ddkain@ucsd.edu Steve Benowitz, 619-543-6163, sbenowitz@ucsd.edu;