Stem Cell Agency Board Funds More than $61 million in Cancer, Sickle Cell Disease and Vision Loss and Sets New Focus

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San Francisco, CA – Reflecting the progress being made in moving the most promising therapies out of the lab and into clinical trials, the state stem cell agency, the California Institute for Regenerative Medicine (CIRM), today voted to approve $61 million in funding for its third round of Disease Team awards.

This round of awards targets diseases like leukemia and other solid tumor cancers – such as breast and prostate – that have not responded to conventional treatment. Other diseases targeted include sickle cell disease and macular degeneration, one of the leading causes of blindness in the elderly.

"Funding this research to find a cure for sickle cell disease would mean that I am the last generation of mothers in my family to spend her adult life keeping her child alive," says Adrienne Shapiro, a patient advocate whose daughter has the potentially deadly blood disorder. "It means I will no longer have to watch my bright, beautiful, brave girl as this horrible painful disease ravages her body and the pain medicines impair her mind. It means I will no longer have to keep fighting for her while the weight of the psychological and social issues surrounding the treatment of Sickle Cell wears away at her spirit."

"The goal of the Disease Team award is to help accelerate the development of new therapies," says Alan Trounson, Ph.D., President of the agency. "I think this is the sharp end of the CIRM program – we need to get therapies into clinical trials. The scientists are working together as teams to demonstrate the safety and efficacy of their products that have evolved from discoveries in the laboratory. What's impressive about this series of awards is that five of the six successful applications are for the continuation of work we had previously funded. It's a reflection of the importance of continuity of funding, enabling scientists to keep their teams together and move their work forward as quickly as possible."

The Disease Team awards are designed to encourage multidisciplinary teams of researchers from academic institutions, medical centers and industry to work together and to develop new treatments for a broad range of therapies. The recipients were selected from 14 applications, all of which were reviewed by an independent group of internationally renowned scientists.

"Three of these successful studies arose as collaborations between California scientists and those in other countries 4 years ago," observed Trounson. "Funding agencies in Canada and the United Kingdom supported work in those countries, and CIRM funded the teams in California. This international teamwork has been crucial to the projects' success."

The funding was approved by the stem cell agency's governing Board, the Independent Citizen's Oversight Committee (ICOC), at a two-day meeting in Los Angeles.

"I think this round of funding speaks volumes for the quality of the work we support," says Jonathan Thomas, Ph.D., J.D., Chairman of the Board. "Many of these projects are ones we have previously funded so to have our outside expert reviewers look at them and recommend continued investment in this research shows we are on the right path."

The Board also discussed the recommendations of the President's Scientific Advisory Board (SAB) that were presented at the last ICOC meeting in October. The SAB was composed of experts in the scientific, clinical, ethical, industry and regulatory aspects of stem cell biology to give the President advice and suggestions on strategic priorities for future research and how best to allocate existing funds.

Based on the report the Board decided to create an Accelerated Development Pathway, putting aside $200 million in a strategic reserve, to be used in helping select projects already funded by the agency speed up their development and move them through the clinical trial approval process as quickly and safely as possible. The money would be used for research that is already in or close to a clinical trial but needs additional funds to get through a Phase II trial to prove the therapy is both safe and effective.

In other recommendations by the SAB the Board voted to discontinue further funding for the Shared Laboratories award, to continue funding for the Early Translation program, and to continue funding, but at a reduced level, for the Basic Research program. The Board also asked staff to provide them with a detailed analysis of the effectiveness of the Training Grants, Bridges and Creativity programs, to

determine if they wanted to continue funding them.

“We deeply appreciate the thought and consideration the Board put into making these decisions as they will have long-term implications for the way the agency works,” said Trounson. “Setting priorities is never easy but this gives us a much clearer sense of where we want to go and where we want to focus our resources and energies.”

The Board also took the opportunity to honor two former ICOC members, Leeza Gibbons, the patient representative for Alzheimer’s disease, and Jon Shestack, the patient representative for mental health.

“You never really say goodbye to people who are as passionate and committed to this work as Leeza and Jon are,” says Thomas. “We know they will both remain engaged in this work, but we shall miss their presence on the Board. They enriched our work with their compassion, their intellect and their unfailing focus on the agency’s goal of finding cures and treatments for deadly diseases.”

Funded projects

Application	Researcher	Institution	ICOC committed Funding
DR3-06924	Thomas Kipps	UCSD	$4,179,600
DR3-06965	Irv Weissman	Stanford	$12,726,396
DR3-07438	Mark Humayun	USC	$18,922,665
DR3-07067	Dennis Slamon	UCLA	$6,924,317
DR3-07281	Peter Belafsky	UC Davis	$4,440,000
DR3-06945	Donald Kohn	UCLA	$13,935,441
		Total	**$61, 128,419**

Project descriptions

Thomas Kipps, University of California San Diego, Leukemia

The group has a prior Disease Team Award to test drug candidates already in use or under evaluation in the pharmaceutical industry to see which are most effective at eradicating the leukemia stem cells in a lab dish. With this award they intend to test a drug discovered through the previous award in clinical trials. Project details.

Irv Weissman, Stanford, Leukemia

This team has found a protein on the surface of leukemia stem cells that protects those cells from elimination by the patient’s own immune system. They call this protein a “don’t eat me” signal. In a previous Disease Team Award they created an antibody that blocks that protein and makes the leukemia stem cell available to be attacked and destroyed by the immune system. With this award they intend to begin testing their antibody in clinical trials. Project details.

Mark Humayun, University of Southern California, macular degeneration

In a previous Disease Team Award, this team proposes to use embryonic stem cells to produce the support cells, or RPE cells, needed to replace those lost in age-related macular degeneration. Because these cells exist in a thin sheet in the back of the eye, they are assembling these sheets in the lab by growing the RPE cells on synthetic scaffolds. These sheets would be surgically implanted into the eye. With this award they plan begin testing their approach in a clinical trial. Project details.

Dennis Slamon, University of California Los Angeles, solid tumors

In a previous Disease Team Award, this team identified a drug that kills cancer stem cells from the ovary, colon and brain in the lab dish. With this award they plan to start testing their drug in a clinical trial. Project details.

Peter Belafsky, University of California Davis, airway blockage

The team uses a trachea from a cadaver as a scaffold, removing the soft tissue cells and then seeding the remaining scaffold with two

types of stem cells from the patient. That construct is grown in a bioreactor until it is ready for transplant in the patient. The team has already used the procedure through the European compassionate use exemption in five dying patients, saving three of their lives. They plan to use this award to do tests in non-human primates to better understand the role of each type of stem cell used to seed the scaffold. Projet details.

Donald Kohn, University of California Los Angeles, sickle cell disease

This team of researchers plans to remove bone marrow cells from people with sickle cell disease and fix the genetic mutation that causes the disease. The team will then reintroduce the new cells into the patient. Those cells will then generate new, healthy blood cells. In a previous Disease Team Award the team showed that the technique could be effective. In this project they intend to start testing their approach in clinical trials. Project details.

About CIRM: CIRM was established in November 2004 with the passage of Proposition 71, the California Stem Cell Research and Cures Act. The statewide ballot measure, which provided $3 billion in funding for stem cell research at California universities and research institutions, was overwhelmingly approved by voters, and called for the establishment of an entity to make grants and provide loans for stem cell research, research facilities, and other vital research.
