Stem Cell Agency Commits $150 Million To Develop New Therapies

Posted: July 27, 2012

San Francisco, CA – California's stem cell agency, the California Institute for Regenerative Medicine (CIRM) today approved $150 million in new funding to help move promising stem cell-based therapies from the laboratory research phase to clinical trials in people.

For Huntington's disease the funding is enormously important in helping researchers at UC Davis do the most advanced clinical study to date for this untreatable and uniformly fatal disease.

"People are hopeful, truly hopeful for the first time," says Judy Roberson, a Huntington's disease patient advocate. "This is a nightmarish, cruel disease in every way but now, thanks to CIRM, we are turning the dream of a stem cell therapy trial into a reality. Research means hope for people with this disease, but research costs money. CIRM has given us all hope."

The grants, up to $20 million per applicant, go to teams of researchers in both academia and industry who have been working on projects that represent the best possible chances of producing therapies for deadly and disabling diseases and disorders. Those diseases include Huntington's disease, metastatic melanoma, osteoporosis, critical limb ischemia, spinal cord injury, ALS (Lou Gehrig's disease) and cardiovascular disease.

"Everything we do in this innovative Disease Team Program is focused on getting good science converted to productive treatments for patients," says Alan Trounson, PhD, President of CIRM. "These awards reflect and highlight our commitment to identifying the most promising stem cell research and supporting it for the time needed to show both the safety and effectiveness of therapy, with an ultimate goal of producing a new treatment that is approved by the FDA for clinical application."

The funding is part of the stem cell agency's Disease Team II awards which 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 21 applications, all of which were reviewed by an independent group of internationally renowned scientists

"This round of funding highlights how we try to support promising research all the way through from basic science to clinical trials," says Jonathan Thomas, PhD, JD, Chair of the Governing Board of CIRM. "For example, Antoni Ribas at UCLA was previously awarded a New Faculty award by CIRM (http://www.cirm.ca.gov/content/stem-cells-immune-system-regeneration-fig...), and now he has been given a Disease Team award to further advance his work in metastatic melanoma.

In the case of five awards—DR2A-05735 addressing heart disease, DR2A-05426 addressing muscular dystrophy, DR2-05416 addressing Alzheimer's disease, DR2-05352 addressing breast cancer and DR2-05739 addressing retinitis pigmentosa—the Board determined that there was enough new information to merit sending the applications back for further expert analysis. The results of that analysis will be brought back before the Board for its consideration at a later date.

The teams that are being given the funding are expected to file a request to begin clinical trials or to complete phase I or II clinical trials within four years. Five of the teams propose to finish a clinical trial within the period of the award.

Two of CIRM's 20 collaborative funding partners around the world will also be contributing to this round of projects. An investigator at the National Institutes of Health will be a partner Principal Investigator with the University of California, Los Angeles team developing a therapy for metastatic melanoma; and the Andalusian Initiative for Advanced Therapies in Spain will be providing $1.6 million to researchers there to collaborate with the team at UC Davis working on a therapy for limb ischemia.

The board also approved the fourth Research Leadership Award to foster the recruitment of Andrew McMahan from the Harvard Stem Cell Institute to the University of Southern California's Broad Center for Regenerative Medicine and Stem Cell Research. He plans to use the $5.7 million award to study ways to repair and regenerate kidney tissue.

CIRM funds late-stage research projects moving toward potential therapies in 37 diseases. The full list and descriptions of the projects can be found on the CIRM website.

Funded Projects

Disease Team Therapy Development Awards

Number	PI	Title	Institution	Committed funds
DR2A-05415	Vicki Wheelock	MSC engineered to produce BDNF for the treatment of Huntington's disease	University of California, Davis	$18,950,061
DR2A-05309	Antoni Ribas	Genetic Re-programming of Stem Cells to Fight Cancer	University of California, Los Angeles	$19,999,563
DR2A-05302	Nancy Lane	Treatment of osteoporosis with endogenous Mesenchymal stem cells	University of California, Davis	$19,999,867
DR2A-05423	John Laird	Phase I study of IM Injection of VEGF-Producing MSC for the Treatment of Critical Limb Ischemia	University of California, Davis	$14,184,595
DR2A-05736	Nobuko Uchida	Neural stem cell transplantation for chronic cervical spinal cord injury	StemCells, Inc.	$20,000,000
DR2A-05394	Robert Robbins	Human Embryonic Stem Cell-Derived Cardiomyocytes for Patients with End Stage Heart Failure	Stanford University	$19,999,899
DR2A-05320	Clive Svendsen	Progenitor Cells Secreting GDNF for the Treatment of ALS	Cedars-Sinai Medical Center	$17,842,617
DR2A-05365	Judith Shizuru	A monoclonal antibody that depletes blood stem cells and enables chemotherapy free transplants	Stanford University	$20,000,000
Total				$150,976,602

Research Leadership Awards

LA1-06536	Andrew McMahon	Repair and regeneration of the nephron	University of Southern California	$6,718,471

Disease Team Project Descriptions

Huntington's Disease: Vicki Wheelock, University of California, Davis
This research team plans to use bone marrow derived mesenchymal stem cells to deliver a growth factor to patients' damaged and endangered nerves. The factor they have chosen, called BDNF, has been shown to be effective in laboratory studies in reducing nerve cell death and improving the function of nerves. During this project they will finalize laboratory tests and begin a phase 1 clinical trial in patients to test the safety of the approach. More information about Huntington's disease.

Malignant Melanoma: Antoni Ribas, University of California, Los Angeles
This team plans to use gene modification technology to create two types of cells that have T-Cell Receptors that specifically target melanoma. They hope to provide patients with modified mature immune cells that are instantly ready to seek out the tumor, as well as blood-forming stem cells that have been modified so that they can produce a perpetual supply of immune cells capable of recognizing melanoma. They plan to conduct the pre-clinical testing required by the FDA as well as the phase one human clinical trial that will look at the therapy's safety. More information about melanoma.

Osteoporosis: Nancy Lane, University of California, Davis
This team proposes to increase the effectiveness of a patient's own bone-forming mesenchymal stem cells. They have developed a drug that directs the stem cells to go to the bone surface and form new bone. During the first phase of the grant they will be working to ensure this drug meets the highest quality standards required by the FDA, and testing it in the lab to make sure it is both safe and shows some signs of being effective. During the second part of the project they plan to conduct a phase 1 trial to gauge safety, first in postmenopausal women and then in both women and men. More information about osteoporosis.

Limb Ischemia: John Laird, University of California, Davis

This team plans to use gene modification techniques to insert a gene for a growth factor called VEGF into a type of stem cell found in bone marrow called mesenchymal stem cells (MSC). VEGF, which is known to stimulate blood vessel growth, has been tried as a treatment for CLI in the past with very limited impact, possibly because the protein does not stay where it is needed long enough. By putting the growth factor into MSCs, which naturally home to inflammation like that in the blocked vessels, the Davis team hopes to get local production of VEGF long enough to stimulate sufficient new vessels to be clinically beneficial. They plan to spend the first year completing the animal testing needed to apply to the FDA to begin a phase 1 trial in the second phase of the grant, which would test the safety of the procedure in humans. More information about limb ischemia.

Spinal Cord Injury: Nobuko Uchida, Stem Cells Inc.

This research team plans to lay the groundwork for the first clinical trial using stem cells to treat spinal cord injuries in the neck. The team is already using stem cells to treat injuries in the back and plan to use these same cell lines - and conduct the added tests needed to get FDA approval - to begin testing patients with neck injuries. They are seeking approval to treat both recently injured patients and people who have had their paralysis for months or years. More information about spinal cord injury.

Heart Disease: Robert Robbins, Stanford School of Medicine

This team plans to turn embryonic stem cells into what are called cardiomyocites, the kind of cells that can become heart muscle. They plan to develop methods for producing sufficient quantities for clinical therapy and to do all the laboratory work and preliminary testing needed to gain FDA approval of a clinical trial by the close of the grant. They are proposing to carry out a trial with patients who have disease that is so advanced that they are on a waiting list for heart transplants. More information about heart disease.

ALS (Lou Gehrig's Disease): Clive Svendsen, Cedars-Sinai Medical Center

This team plans to modify neural stem cells as a possible therapy for ALS. The genetically modified stem cells will produce a protein that they hope will protect those cells and also helps protect any remaining nerve cells in the brain that are not already damaged. The team intends to inject those modified stem cells into the brain where they will replace the type of cell—called astrocytes—that are damaged in the disease. They will then run a series of tests to make sure the cells are safe, and to determine if they really do have a protective effect. More information about ALS.

Severe Combined Immune Deficiency: Judith Shizuru, Stanford School of Medicine

This team proposes to replace SCID patients' dysfunctional immune cells with healthy ones using a safer form of bone marrow transplant (BMT). They plan to eliminate the bad cells with an antibody, a protein, that very specifically targets and eliminates blood forming stem cells. If successful, the procedure could open up similar BMT therapies to patients with other auto-immune diseases such as multiple sclerosis, lups or diabetes that are generally not candidates for BMT currently. These diseases, while debilitating, are not immediately life-threatening and generally don't warrant the risks involved in BMT the way it is done today. More information about SCID.

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 opportunities. A list of grants and loans awarded to date may be seen here: http://www.cirm.ca.gov/for-researchers/researchfunding.

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