CIRM Board Champions Fight Against Rare Diseases Affecting Children

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San Francisco, CA – Five fatal conditions that begin in early childhood - Duchenne muscular dystrophy, severe combined immunodeficiency, Canavan disease, Tay-Sachs and Sandhoff disease - were among nine programs awarded more than $44.5 million by the governing Board of CIRM, the California Institute for Regenerative Medicine.

Duchenne muscular dystrophy (DMD) is a rapidly progressive form of muscular dystrophy that affects mostly boys and leads to loss of muscle function. It is often diagnosed before age 6 and few individuals live beyond their 30's. Heart failure is the most common cause of death and there are no effective treatments for this aspect of DMD.

The CIRM Board approved $3.4 million for Los Angeles-based company Capricor to carry out a Phase 1/2 clinical trial using its CAP-1002 product. This is an "off-the-shelf" product that uses cardiosphere, or heart-derived stem cells, to help repair the damage to the heart caused by DMD.

"There can be nothing worse than for a parent to watch their child slowly lose a fight against a deadly disease," says C. Randal Mills, Ph.D., President and CEO of CIRM. "Many of the programs we are funding today are focused on helping find treatments for diseases that affect children, often in infancy. Because many of these diseases are rare there are limited treatment options for them, which makes it all the more important for CIRM to focus on targeting these unmet medical needs."

Researchers at the University of California, San Francisco were also approved for $4.3 million to complete the preclinical work needed to develop a therapy for children with severe combined immunodeficiency (SCID) caused by a defect in the ART gene. Children born with SCID have severely weakened immune systems making it hard for them to fight infections. The standard treatment is a bone marrow transplant but for children with the ART gene defect, who don't have a matched sibling bone marrow donor, this can put them at increased risk for potentially deadly-complications. The UCSF team hopes to use the patient's own blood-forming stem cells, genetically modified to correct for the ART defect, to treat the children.

The CIRM Board also approved seven awards worth $36.7 million under it's Translation program. The goal of this program is to help promising stem cell-based projects complete the research necessary to advance either into a clinical trial or, in the case of a medical device, for broader use.

Among the projects approved are:

- Research to help develop a new treatment for Canavan disease, a fatal brain disorder that often begins in infancy
- Creating brain cells from embryonic stem cells to help people with ALS or Lou Gehrig's disease
- Using a combination of gene therapy and blood stem cells to develop a therapy for Tay-Sachs and Sandhoff disease

"The goal of our Translation program is to support the most promising stem cell-based projects and to help them accelerate that research out of the lab and into the real world, such as a clinical trial where they can be tested in people," says Jonathan Thomas, Ph.D., J.D. Chair of the CIRM Board. "The projects that our Board approved today are a great example of work that takes innovative approaches to developing new therapies for a wide variety of diseases."

The successful applications are:

Application	Researcher	Institution	ICOC Committed funding

TRAN1-08525 Canavan disease	Yanhong Shi	City of Hope	$7,377,384
TRAN1-08471 Enteric neuropathies	Tracy Grikscheit	Children's Hospital Los Angeles	$7,139,913
TRAN1-08635 Cancer stem cells	William Murphy	U.C. Davis	$2,368,818
TRAN1-08561 Diabetes	Felicia Pagliuca	Semma Therapeutics	$5,000,000
TRAN1-08519 Tay-Sachs & Sandhoff disease	Joseph Anderson	U.C. Davis	$883,174
TRAN1-08552 ALS	Larry Goldstein	U.C. San Diego	$6,349,278
TRAN1-08533 Cancer	Lili Yang	U.C. Los Angeles	$7,659,309

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov.

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