Stem Cell Agency Focuses Almost $38 Million on Colorectal Cancer, a Deadly Childhood Disorder and High Blood Pressure in the Lungs

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Oakland, CA – Colorectal cancer is the second leading cause of cancer death in the U.S., claiming more than 49,000 lives a year. Only around 10 percent of patients survive longer than five years, and for the 40 percent of patients whose cancer has a mutation in the KRAS gene, survival can often be measured in months. Now a new CIRM-funded clinical trial could change that.

That program is one of three new clinical trials that the CIRM governing Board approved today.

“In everything we do there is a real sense of urgency, because lives are at stake,” says C. Randal Mills, Ph.D., President and CEO of CIRM. “Our Board’s support for these programs highlights how every member of the CIRM team shares that commitment to moving the most promising research out of the lab and into patients as quickly as we can. These are very different projects, but they all share the same goal, accelerating treatments to patients with unmet medical needs.”

In the colorectal cancer program, Forty Seven Inc. has been awarded $10.2 million to use a combination one-two punch to attack the cancer in patients with the KRAS genetic mutation. Cancer stem cells, which help fuel the growth of the disease, have a receptor on their surface called CD47, dubbed by one researcher as the “don’t eat me” gene because it tricks the immune system cells responsible for getting rid of tumors into not doing their job. Forty Seven Inc. has an antibody that blocks the CD47 receptor, turning the cancer into a target for both the immune system and an anti-cancer drug called cetuximab, the second element in the one-two punch.

In vivid contrast to colorectal cancer, Adenosine Deaminase Severe Combined Immune Deficiency (ADA-SCID) is a rare form of a rare disease. The condition is also known as “bubble baby” disease because it leaves children with virtually no immune protection from infections and in the past some children were placed in sterile plastic bubble-like environments to protect them. The condition can be fatal within the first years of life or if left

untreated.
A team from the University of California, Los Angeles, led by Don Kohn, was awarded $20 million to take a patient's own blood stem cells, genetically modify them to correct the defect that causes ADA-SCID, and then reinfuse those cells back into the patient. The modified stem cells will then, hopefully, create a new healthy and functioning blood and immune system.
Kohn has already used a similar method to cure 23 SCID children. This latest approach has been approved by the Food and Drug Administration (FDA) for a Phase 2 clinical trial.
The CIRM Board also approved $7.35 million for a team at Cedars-Sinai Medical Center to test the use of stem cells to treat pulmonary hypertension, caused by high blood pressure in the arteries that go from the heart to the lungs. This is a chronic, life-changing disease that can ultimately lead to heart failure. The team wants to use cells derived from heart stem cells, also known as cardiospheres, to reduce inflammation in the arteries and reduce blood pressure.

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

LLS EXPANDS INNOVATIVE THERAPY ACCELERATION PROGRAM WITH NOVEL IMMUNOTHERAPY

LLS Commits $4 Million to Forty Seven Inc.'s Cancer Program

Rye Brook, NY (April 5, 2017) - The Leukemia & Lymphoma Society (LLS) continues to advance the promising field of immunotherapy research, harnessing the body's own immune system to fight cancer, with a $4 million funding commitment in an investigational therapy being developed by Forty Seven Inc. for lymphoma patients.

There are approximately 630,000 patients in the United States living with non-Hodgkin lymphoma (NHL), a diverse group of blood cancers that begin in the body's lymphatic system. LLS's investment will support Forty Seven's clinical trial using an antibody therapy (Hu5F9-G4) aimed at treating two types of NHL - diffuse large B-cell lymphoma (DFBCL) and follicular lymphoma (FL). DLBCL represents approximately 30 percent of NHL patients, with 60 percent of patients surviving five years after diagnosis; however, more than one-third of patients either relapse or do not respond to therapy. Approximately 25 percent of NHL patients are diagnosed with FL, a slow-growing form of the disease. While most patients with FL respond to initial therapy, more than 70 percent are diagnosed with advanced stage disease and are considered incurable.

The novel drug will be tested in combination with the FDA-approved rituximab, already part of standard treatment for several types of NHL. The therapy is directed against CD47, a protein that provides a "don't eat me" signal to the immune system and blocks the ability of immune cells called macrophages to devour those cancer cells. The combination Hu5F9-G4 and rituximab displayed synergy in preclinical animal models of NHL.[1]

According to LLS President and CEO Louis J. DeGennaro, Ph.D., "LLS welcomes this collaboration with Forty Seven to advance this novel immunotherapy approach. It's critical that LLS identifies and acts on the most promising areas of cancer research, and in Forty Seven we have found a partner who is as committed to accelerating cures as we are."

Forty Seven was founded in 2015 by Stanford University researchers Irv Weissman, M.D., and Ravi Majeti, M.D., Ph.D., both of whom have been recipients of LLS grants

supporting their early work targeting CD47. Forty Seven has licensed the therapy from Stanford.

LLS is supporting the collaboration through its Therapy Acceleration Program ® (TAP), a strategic initiative to partner directly with biotechnology companies to help accelerate the development of promising therapies. There are currently 16 projects supported through LLS's TAP portfolio.

LLS supports a wide array of immunotherapy programs at major cancer research centers such as Memorial Sloan Kettering Cancer Center, Dana-Farber Cancer Institute and Fred Hutchinson Cancer Research Center, as well as through TAP partnerships. LLS is currently committed to more than $40 million to study novel immunotherapy approaches to controlling blood cancers.

About The Leukemia & Lymphoma Society

The Leukemia & Lymphoma Society® (LLS) is the world's largest voluntary health agency dedicated to blood cancer. The LLS mission: Cure leukemia, lymphoma, Hodgkin's disease and myeloma, and improve the quality of life of patients and their families. LLS funds lifesaving blood cancer research around the world, provides free information and support services, and is the voice for all blood cancer patients seeking access to quality, affordable, coordinated care.

Founded in 1949 and headquartered in Rye Brook, NY, LLS has chapters throughout the United States and Canada. To learn more, visit LLS.org. Patients should contact the Information Resource Center at (800) 955-4572, Monday through Friday, 9 a.m. to 9 p.m. ET.

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[1] (Pre-Clinical Development of a Humanized Anti-CD47 Antibody with Anti-Cancer Therapeutic Potenti PLoS One. 2015 Sep 21;10(9)