CIRM Board Approves Funding for New Clinical Trials in Solid Tumors and Pediatric Disease

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Oakland, CA – On April 29th, the governing Board of the California Institute for Regenerative Medicine (CIRM) awarded two grants totaling $11.15 million to carry out two new clinical trials. These latest additions bring the total number of CIRM funded clinical trials to 53.

$6.56 Million was awarded to *Rocket Pharmaceuticals, Inc.* to conduct a clinical trial for treatment of infants with Leukocyte Adhesion Deficiency-I (LAD-I)

LAD-I is a rare pediatric disease caused a mutation in a specific gene that affects the body's ability to combat infections. As a result, infants with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Those that survive infancy experience recurrent severe infections, with mortality rates for severe LAD-I at 60-75% prior to the age of two and survival very rare beyond the age of five.

Rocket Pharmaceuticals, Inc. will test a treatment that uses a patient's own blood stem cells and inserts a functional version of the gene. These modified stem cells are then reintroduced back into the patient that would give rise to functional immune cells, thereby enabling the body to combat infections.

The award is in the form of a CLIN2 grant, with the goal of conducting a clinical trial to assess the safety and effectiveness of this treatment in patients with LAD-I.

This project utilizes a gene therapy approach, similar to that of three other clinical trials funded by CIRM and conducted at UCLA by Dr. Don Kohn, for X-linked Chronic Granulomatous Disease, an inherited immune deficiency "bubble baby" disease known as ADA-SCID, and Sickle Cell Disease.

An additional $4.59 million was awarded to Dr. Theodore Nowicki at UCLA to conduct a clinical trial for treatment of patients with sarcomas and other advanced solid tumors. In 2018 alone, an estimated 13,040 people were diagnosed with soft tissue sarcoma (STS) in the United States, with approximately 5,150 deaths. Standard of care treatment for sarcomas typically consists of surgery, radiation, and chemotherapy, but patients with late stage or recurring tumor growth have few options.

Dr. Nowicki and his team will genetically modify peripheral blood stem cells (PBSCs) and peripheral blood monocular cells (PBMCs) to target these solid tumors. The gene modified stem cells, which have the ability to self-renew, provide the potential for durable effect.

This award is also in the form of a CLIN2 grant, with the goal of conducting a clinical trial to assess the safety of this rare solid tumor treatment.

This project will add to CIRM's portfolio in stem cell approaches for difficult to treat cancers. A previously funded a clinical trial at UCLA uses this same approach to treat patients with multiple myeloma. CIRM has also previously funded two clinical trials using different approaches to target other types of solid tumors, one of which was conducted at Stanford and the other at UCLA. Lastly, two additional CIRM funded trials conducted by City of Hope and *Poseida Therapeutics, Inc.* used modified T cells to treat brain cancer and multiple myeloma, respectively.

"CIRM has funded 23 clinical stage programs utilizing cell and gene medicine approaches" says Maria T. Millan, M.D., the President and CEO of CIRM. "The addition of these two programs, one in immunodeficiency and the other for the treatment of malignancy, broaden the scope of unmet medical need we can impact with cell and gene therapeutic approaches."

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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