For immediate release:

Contact: Maria Bonneville at 415-740-8623 or mbonneville@cirm.ca.gov

Stem Cell & Gene Therapy Agency Funds Clinical Trial to Make Cancer Therapy Safer and Less Toxic

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South San Francisco, CA – Blood stem cell transplantation following high dose chemotherapy is standard of care and potentially curative for aggressive forms of lymphoma. However, this treatment regimen is limited by severe toxicity and life-threatening complications due to delayed recovery of the blood system and vascular related damage of multiple organs.

Today the governing Board of the California Institute for Regenerative Medicine (CIRM) funded a Phase 3 clinical trial to support development of a safer, more tolerable alternative.

This brings the number of clinical trials funded by CIRM to 86.

The Board awarded $15,000,000 to Dr. Paul Finnegan and Angiocrine Bioscience to test AB-205, human endothelial cells engineered to express a pro-survival factor. Prior data suggest that, in the setting of chemotherapy and stem cell transplantation, AB-205 cell therapy can accelerate the recovery of the blood system and protects from toxicity by enhancing the recovery from vascular damage. AB-205 is being

studied in a Phase 3 trial in adults with lymphoma undergoing high-dose chemotherapy and autologous blood stem cell transplant.

“If successful, this approach can overcome hurdles to the success of chemotherapy and blood stem cell transplantation for the treatment of advanced blood cancer,” says Dr. Maria T. Millan, President and CEO of CIRM. “This Phase 3 trial is the culmination of preclinical research and the initial clinical trial previously funded by CIRM.”

The CIRM Board awarded $3,999,113 to Dr. David Stover and Nammi Therapeutics to complete manufacture and testing needed to gain FDA permission for a clinical trial for multiple myeloma and advanced solid tumors. The therapy QXL 138AM, a “masked immunocytokine,” binds to and kills the tumor by activating the immune system.

The CIRM Board also awarded $4,048,253 to Dr. Joseph Anderson and his team at UC Davis to develop a blood stem cell gene therapy for the treatment of Tay-Sachs disease. Tay-Sachs disease is a rare genetic disorder where a deficiency in the hexosaminidase A enzyme results in excessive accumulation of certain fats in the brain and nerve cells and causes progressive dysfunction. The UC Davis team will genetically modify the patient’s own blood stem cells to restore the hexosaminidase enzyme that is missing in the disease. The goal is to complete safety studies and to apply to the US Food and Drug Administration for an Investigational New Drug (IND), the authorization needed to begin a clinical trial in people. This work is a continuation of a TRAN 1 award that the team received.

Finally, the CIRM Board awarded $6,000,000 to Dr. Karin Gaensler at the University of California, San Francisco (UCSF) to support development of a vaccine for the blood cancer acute myelogenous leukemia (AML). To develop the cancer vaccine, Dr. Gaensler and her team will engineer the patient’s blood stem cells to maximize stimulation of leukemia-specific killing activity and reintroduce engineered cells back to the patient to target and kill residual leukemia stem cells. This approach holds the potential for long-term effectiveness as it targets both AML blasts and leukemic stem cells that are often the source of relapse. This award which is a continuation of a CIRM TRAN 1 grant will support the manufacture of the vaccine and the completion of late-stage testing and preparation needed to apply to the US Food and Drug Administration (FDA) for permission to begin a clinical trial.

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CALIFORNIA'S STEM CELL AGENCY

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 $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

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