CIRM Invests $10 Million in Clinical Trial to Improve Dialysis and $13.6 Million for Promising Early Research Projects

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Oakland, CA – Nearly half a million Americans with kidney disease are on dialysis, a life-supporting treatment that filters out impurities and cleans the blood. Today the California Institute for Regenerative Medicine approved $10 million to help fund a clinical trial testing a device that could make life easier, and safer for many of those on dialysis.

The Phase 3 clinical trial is being run by Humacyte Inc. which is testing a bioengineered vein needed by people undergoing hemodialysis, the most common form of dialysis. The vein – also known as a human acellular vessel or HAV – is implanted in the arm and used to carry the patient's blood to and from an artificial kidney that removes waste from the blood. Current synthetic versions of this device have many problems, including clotting, infections and rejection. Humacyte has created bioengineered blood vessels that appear to be both safer and more durable than existing HAVs. In addition, over time the patient's own stem cells start to populate the bioengineered vein, in effect making it part of the patient's own body.

"This approach has the potential to significantly improve our ability to care for people with kidney disease," says C. Randal Mills, Ph.D., the President and CEO of CIRM. "Being able to reduce infections and clotting problems, and increase the consistency of care hemodialysis patients get, would meaningfully impact the quality of their lives."

In addition, the CIRM Board approved $3.9 million to develop and test an implantable cell therapy for people with a high-risk form of type 1 diabetes. There are more than 100,000 people in the US with such severe type 1 diabetes that, despite the availability and use of insulin, are at constant risk of hospitalization and even death.

The cell therapy, known as PEC-Direct, is being developed by ViaCyte and uses pancreatic progenitor cells which have the ability to turn into all the endocrine cell types found in the pancreas, the organ damaged in diabetes. The pancreatic progenitor cells are delivered in a device that is implanted under the skin where it allows direct vascularization, connecting the cells in the PEC-Direct product to the patient's blood circulatory system. This vascularization enables the cells to monitor blood sugar levels, detect when they are too high or low, and secrete insulin and other hormones that will restore and maintain blood sugar at healthy levels.

CIRM is also funding a clinical trial that ViaCyte is currently performing, using a more broadly applicable form of this cell therapy, known as PEC-EnCap (formerly called VC-01). Rather than using an open delivery system that allows direct vascularization of the cells, PEC-EnCap relies on the Encaptra® Drug Delivery System to protect the cells from the immune system. Both PEC-Direct and PEC-EnCap deliver the same stem cell-derived pancreatic progenitor cell product, known as PEC-01 cells.

Because PEC-Direct utilizes a more open device than PEC-EnCap it also will require those receiving it to take chronic immune suppression. For that reason, PEC-Direct is being developed only for patients with type 1 diabetes that are at highest risk for acute complications such as severe hypoglycemic events, and/or those with hypoglycemia unawareness, and for those who are already on immune suppression to support an organ, such as a kidney transplant.

The Board also approved several awards under it's Discovery Quest program. The Quest awards promote the discovery of promising new stem cell-based technologies that could be translated to enable broad use and ultimately, improve patient care.

Application	Title	Institution	ICOC Committed funding
DISC2-09098	Use of Human iPSC-derived Endothelial Cells for Calcific Aortic Valve Disease Therapeutics	J. David Gladstone Institutes Principal investigator; D. Srivastava	$2,400,048

DISC2-08874	Novel Rejuvenated T Cell Immunotherapy for Lung Cancer	Stanford University Principal investigator: H. Nakauchi	$2,217,264
DISC2-08824	CRISPR/Cas9 nanoparticle enabled therapy for Duchenne Muscular Dystrophy in muscle stem cells	University of California, Los Angeles Principal investigator: A. Pyle	$2,150,400
DISC2-09123	Immunotherapy for HIV infection using engineered hematopoietic stem/progenitor cells	California Institute of Technology Principal investigator: D. Baltimore	$1,586,934
DISC2-09032	MSC delivery of an artificial transcription factor to the brain as a treatment for Angelman Syndrome	University of California, Davis Principal investigator: D. Segal	$1,087,572
DISC2-09073	Autologous cell therapy for Parkinson's disease using iPSC-derived DA neurons	Scripps Research Institute Principal investigator: J. Loring	$2,354,226
DISC2-08982	Scalable, Defined Production of Oligodendrocyte Precursor Cells to Treat Neural Disease and Injury	University of California, Berkeley Principal investigator: D. Schaffer	$1,848,462

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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