
Rare Disease Gets Big Boost from California's Stem Cell Agency

Posted: January 30, 2019

Oakland, CA – If you were looking for a poster child for an unmet medical need Huntington's disease (HD) would be high on the list. It's a devastating disease that attacks the brain, steadily destroying the ability to control body movement and speech. It impairs thinking and often leads to dementia. It's always fatal and there are no treatments that can stop or reverse the course of the disease. Today the Board of the California Institute for Regenerative Medicine (CIRM) voted to support a project that shows promise in changing that.

The Board voted to approve \$6 million to enable Dr. Leslie Thompson and her team at the University of California, Irvine to do the late stage testing needed to apply to the US Food and Drug Administration for permission to start a clinical trial in people. The therapy involves transplanting stem cells that have been turned into neural stem cells which secrete a molecule called brain-derived neurotrophic factor (BDNF), which has been shown to promote the growth and improve the function of brain cells. The goal is to slow down the progression of this debilitating disease.

"Huntington's disease affects around 30,000 people in the US and children born to parents with HD have a 50/50 chance of getting the disease themselves," says Dr. Maria T. Millan, the President and CEO of CIRM. "We have supported Dr. Thompson's work for a number of years, reflecting our commitment to helping the best science advance, and are hopeful today's vote will take it a crucial step closer to a clinical trial."

Another project supported by CIRM at an earlier stage of research was also given funding for a clinical trial.

The Board approved almost \$12 million to support a clinical trial to help people undergoing a kidney transplant. Right now, there are around 100,000 people in the US waiting to get a kidney transplant. Even those fortunate enough to get one face a lifetime on immunosuppressive drugs to stop the body rejecting the new organ, drugs that increase the risk for infection, heart disease and diabetes.

Dr. Everett Meyer, and his team at Stanford University, will use a combination of healthy donor stem cells and the patient's own regulatory T cells (Tregs), to train the patient's immune system to accept the transplanted kidney and eliminate the need for immunosuppressive drugs.

The initial group targeted in this clinical trial are people with what are called HLA-mismatched kidneys. This is where the donor and recipient do not share the same human leukocyte antigens (HLAs), proteins located on the surface of immune cells and other cells in the body. Around 50 percent of patients with HLA-mismatched transplants experience rejection of the organ.

In his application Dr. Meyer said they have a simple goal: "The goal is "one kidney for life" off drugs with safety for all patients. The overall health status of patients off immunosuppressive drugs will improve due to reduction in side effects associated with these drugs, and due to reduced graft loss afforded by tolerance induction that will prevent chronic rejection."

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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