Stem Cell Agency invests in clinical trials for life-threatening childhood diseases and a fatal brain disorder

Posted: February 23, 2017

February 23, 2017

Oakland, CA – Rare diseases are often overlooked when it comes to funding research to develop new treatments. Today, however, the governing Board of the California Institute for Regenerative Medicine (CIRM) focused on two rare diseases, approving an investment of more than $18 million to move promising therapies into clinical trials.

Researchers at Cedars-Sinai Medical Center in Los Angeles will get $6.15 million to run a Phase 1 clinical trial for people with Amyotrophic Lateral Sclerosis (ALS) – also known as Lou Gehrig's disease. This disease destroys the nerve cells in the brain and spinal cord that control movement, speech, even breathing. The average life expectancy for someone with ALS is between 2 and 5 years after diagnosis.

The researchers at Cedars-Sinai will use cells called astrocytes that have been specially re-engineered to secrete proteins that can help repair and replace the cells damaged by the disease.

St. Jude Children's Research Hospital is getting $11.9 million to run a Phase I/II clinical trial for x-linked severe combined immunodeficiency disorder (SCID), a catastrophic condition where children are born without a functioning immune system. Because they are unable to fight off infections, many children born with SCID die in the first few years of life.

St. Jude is teaming up with researchers at the University of California, San Francisco (UCSF) to genetically modify the patient's own blood stem cells, hopefully creating a new blood system and repairing the damaged immune system.

This is the first time CIRM has funded work with St. Jude and reflects our commitment to moving the most promising research into clinical trials in people, regardless of whether that work originates inside or outside California.

"While making a funding decision at CIRM we don't just look at how many people are affected by a disease," says C. Randal Mills, Ph.D., President and CEO of CIRM. "We also look at the severity of the disease on the individual and the potential for impacting other diseases. While the number of patients afflicted by these two diseases may be small, their need is great. Additionally, the potential to use these approaches in treating other disease is very real. The underlying technology used in treating SCID, for example, has potential application in other areas such as sickle cell disease and HIV/AIDS."

The Board also voted to invest $12.2 million in a clinical trial for young people 12 to 17, who are newly diagnosed with type 1 diabetes (T1D). Type 1 diabetes is an autoimmune disorder where the body's own immune system attacks the cells that produce insulin, which is needed to control blood sugar levels. If left untreated it can result in serious, even life-threatening, complications such as vision loss, kidney damage and heart attacks.

Researchers at Caladrius Biosciences will take cells, called regulatory T cells (Tregs), from the patient's own immune system, expand the number of those cells in the lab and enhance them to make them more effective at preventing the autoimmune attack on the insulin-producing cells.

The focus is on newly-diagnosed adolescents because studies show that at the time of diagnosis T1D patients usually have around 20 percent of their insulin-producing cells still intact. It's hoped by intervening early the therapy can protect those cells and reduce the need for patients to rely on insulin injections.

David J. Mazzo, Ph.D., CEO of Caladrius Biosciences, says this is hopeful news for people with type 1 diabetes: "We firmly believe that this therapy has the potential to improve the lives of people with T1D and this grant helps us advance our Phase 2 clinical study with the goal of determining the potential for CLBS03 to be an effective therapy in this important indication."

The CIRM Board also approved $2.29 million for the Cellular Biomedicine Group to do the late-stage research needed to get approval from the Food and Drug Administration (FDA) to move their treatment for osteoarthritis of the knee into a clinical trial.

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
