Promising Approach to Curing Spina Bifida Gets $5.6 Million from Stem Cell Agency

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Oakland, CA – Every day in the U.S. four children are born with spina bifida. It is the most common cause of lifelong paralysis and also frequently leads to other serious health problems affecting the bowel and bladder. The impact on families is enormous. A new approach to repairing the defect that causes spina bifida was today awarded $5.66 million by the Board of the California Institute for Regenerative Medicine (CIRM).

In spina bifida the spinal cord doesn't form properly, in many cases leaving a section of it open, exposing tissues and nerves. The current standard of care is surgery, but even this leaves almost 60% of children unable to walk independently. Diana Farmer MD, and Aijun Wang PhD at U.C. Davis will use mesenchymal stem cells, taken from a donor placenta, and place them on a form of synthetic scaffold over the injury site in the womb. Tests in animals show this approach was able to repair the defect and prevent paralysis.

"Spina bifida is a devastating condition for babies born with this disorder and the families who care for them," says Maria T. Millan, MD, President & CEO of CIRM. "CIRM has funded this important work from its earliest stages and we are committed to working with Dr. Farmer's team to moving this work to the stage where it can be tested in patients."

The CLIN1 award will provide funding to enable the UC Davis team to do the final testing and preparations needed to apply to the FDA for permission to start a clinical trial.

Dr. Farmer says she and Dr. Wang, have been working on this approach for more than ten years and are excited about being able to take the next step.

"There were many times of frustration, many times when cell types we explored and worked with didn't work," says Dr. Farmer. "But it's the patients, seeing them, talking to them and working with them, that keeps me motivated to do the science, to keep persevering."

If this therapy is successful it will have a huge economic impact on California, and on the rest of the world. Because spina bifida is a lifelong condition involving many operations, many stays in the hospital and, in some cases, lifelong use of a wheelchair this has a huge financial, and psychological, burden on the family.

"It affects them in so many ways; parents having to miss work or take time off work to care for their child, other children in the family feeling neglected because their brother or sister needs so much attention," says Dr. Farmer. "That's why we are so grateful to CIRM. Because this is a rare disease and finding funding for those is hard. CIRM has been a perfect partner in helping bring this approach, blending stem cell therapy and tissue engineering, together to help these families."

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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