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W.M. Keck Center for
Collaborative Neuroscience

Spring 2017

Dear Friends in the Journey to a Cure,

Flowering crabs are spreading their carpet of blossoms and tulips are peeking through the ground – signs that a new season is on the brink of bursting forth!

In the same way, newspaper headlines raise glimmers of hope and applications to the FDA point to the budding emergence of clinical trials. We all start reading and wondering and asking questions. What to believe? What is a cruel hoax and what is real? When should I sign up, and for what? Where can I get honest answers?

As part of our commitment to find cures for spinal cord injuries, we are dedicated to bringing you the latest information and accurate answers to your questions. In this mailing you will learn about two such opportunities:

- Dr. Young's enclosed letter includes an update on the status of our clinical trials applications around the world.
- The featured speaker at our Summer Open House is Dr. Reggie Edgerton, known as 'the father of spinal cord stimulation'. This July 20th event will give you the details behind the headlines and provide the opportunity for you to ask any and all questions.

The trees and flowers will bloom on their own, but the time will come for you to make decisions. We are excited to provide these occasions for you to gather and assess this important information. We look forward to seeing you in July.

Help Hope Happen,

Patricia

Dr. Patricia Morton, Director
Planning and Development

Much of our research is funded by generous people like you. If you would like to join us in this life-changing work, please return the enclosed slip or donate online at keck.rutgers.edu.

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The Long Journey

Wise Young PhD MD

As I sit on the platform at the Convocation for the Douglass Residential College at Rutgers, my mind is filled with thoughts of our students. I will miss them as they go out into the world where many of them will be contributing to curing spinal cord injury and other conditions that afflict humankind.

Preparing Students for Compassionate Science

At our W. M. Keck Center for Collaborative Neuroscience, we work hard to teach our students not only how to do science but why. Science is about doing good for our world. We teach and practice what I call compassionate science. It is not enough to discover new knowledge, publish, and receive accolades of fellow scientists. It is our responsibility to make sure that discoveries are to help people.

In 1977, as a neurosurgery resident at New York University, I received several NIH grants for spinal cord injury research. After forming the Neurosurgery Laboratory, I asked the chairman if I could come back to finish my residency. He told me I should stay in research because I could do more for patients in the laboratory than in the operating room. At that time, I didn't understand what he meant. Now, after 40 years of research, I finally am beginning to understand. As a neurosurgeon, I might operate on 6,000 patients during my career. But, as a researcher, I can help millions of people with spinal cord injury in my lifetime and beyond.

For much of human history, a cure for spinal cord injury was considered impossible. Over the centuries, nobody held any hope for those who had spinal injury. Even today, movies such as *Me Before You* act like people with spinal cord injury would be better off dead and that families should not have hope.

Then shortly after World War II, with the birth of rehabilitation, hope emerged. With care, people could survive for decades with a fairly normal life. In the 1980's, families began to raise funds for research for treatments. My friend Kent Waldrep held a fundraiser in Texas where they had t-shirts with a picture of a dancing rat. Scientists are making rats walk! In 1996, Christopher Reeve asked me if a cure was possible. I told him that with sufficient resources, it would take seven years. Sadly, it has taken longer. One of my lasting regrets is that Christopher died in 2004 without benefiting from our progress.

Umbilical Cord Blood Mononuclear Cells Restore Walking, Bowel, and Bladder Function

Last year, we published studies from our clinical trials in China which showed that umbilical cord blood mononuclear cells (UCBMNC) transplanted into the spinal cord, coupled with intensive locomotor training, restored walking in 15 of 20 of people with chronic complete spinal cord injury. Over half recovered independent bladder and bowel function. (In previous mailings I shared the details of these trials.) Stemcyte, the world's largest commercial cord blood banking company agreed to sponsor clinical trials to obtain regulatory approval of UCBMNC treatment of chronic spinal cord injury. To get regulatory approval of UCBMNC treatment of spinal cord injury we must do phase III trials.

Therefore, we have proposed trials in India and the U.S. to transplant UCBMNC into 18 patients in each trial and randomize the patients to 6-weeks of lithium or placebo, followed by intensive 6:6:6 locomotor training. In addition to showing whether lithium is beneficial when given with UCBMNC, these trials will confirm that the cells can be safely transplanted in India and the U.S. and patients can be trained to walk 6 hours a day, 6 days a week, for 6 months.

The U.S. FDA met with us in January 2016 and requested two additions. They asked us to transplant human UCBMNC into the spinal cord of immune-incompetent rats to see what happened to the cells and to where they migrated. They also asked us to include a group of patients who received walking training only. We agreed. The rat studies should be completed by mid-June, at which time, we will submit the final application for the clinical trial. If approved, the U.S. trial will start this Fall.

We also proposed a trial of 18 patients in Taiwan to transplant UCBMNC into 9 patients and untethering surgery without transplants in 9 patients, with each group getting 6:6:6 locomotor training. The latter will be offered free UCBMNC transplants at one year. This trial will show whether untethering surgery and walking training without cells restore walking, bowel, and bladder function and whether pre-transplant locomotor training will enhance the effects of transplants.

Hopefully, these trials will be approved this summer so clinical trials can be started in 2017. If the trials show the cells can be transplanted, intensive locomotor training can be done in three countries, and that there is a significant difference between patients who received transplants or only untethering surgery, we will proceed to phase III trials in the U.S., China, India, and Europe in 2019. If the US and India trials show that lithium is not beneficial, we will omit it from the Phase 3 trials to be held in the United States (6 centers), Europe (Norway and Italy), India (3 centers), and China (5 centers). We hope to get worldwide approval by 2020, making the therapy both available and covered by insurance.

Making Umbilical Cord Blood Mononuclear Cells Available and Accessible to Everyone

About 3 million people around the world live with chronic spinal cord injury. Even treating just 10% with UCBMNC would be 300,000 therapies per year. At present, Stemcyte can process just two units of cord blood per day. Also, there are only about 600,000 units stored in public banks around the world. Even if we were allowed to use them, the entire world supply of cord blood would be depleted in two years.

So, for three years, we have worked on a new approach to collecting and processing umbilical cord blood to treat 300,000 people per year. Working with a device developed by Syngen, four umbilical cord blood units can be processed per hour. Equally important, each unit can be frozen into 10 doses of 10 million UCBMNC per unit, so one unit of cord blood now can treat 10 people. We also are implementing a model to collect more cord blood in a way that offers life-saving advantages to newborns. This is being done through a new company , Mononuclear Therapeutics (MT) founded by Ken Giacin and myself. MT will defray the costs of collecting and processing the cord blood by selling the components platelet-rich-plasma (PRP) and red cell fraction (RCF). Once this concept is proven, we will partner with companies in the United States, India, and Europe to do similar collections, to assure that there is enough cord blood to treat everyone with spinal cord injury.

These next three years are critical. I intend to use my shares of MT to set up a trust fund to support future spinal cord injury clinical trials. We also are establishing a *Breakthrough Research Support Fund* to cover the significant costs of bringing even better treatments to trials. You will be hearing more about this and will be invited to participate. We are where we are today because of you. I am hoping you will consider a multi-year pledge so that we can move forward at a faster pace.

The cure is a journey we take together to learn from each other, give each other hope, and ensure that the journey leads to better treatments and care. Thank you for all your support and help.

Wise