JOIN US

We invite you to join our dedicated and exceptional association of scientists, physicians, advocates, educators, philanthropists, public servants, and clergy as we stride forward in unlocking the healing powers that are already inside our own bodies.

To learn more:

Call 212.584.4176 or visit **www.stemforlife.org**

To make a contribution:

Visit **www.stemforlife.org/donate**

or send a check to:

The Stem for Life Foundation
420 Lexington Avenue, Suite 350
New York, NY 10170

To learn more about becoming a Student Ambassador:

Email **studentambassadorprogram@stemforlife.org**

The mission of The Stem for Life Foundation is to raise public awareness about adult stem cells and their therapeutic promise and to support the advancement of adult stem cell research and development.

Stem for Life Foundation

420 Lexington Ave., Suite 350, New York, NY 10170

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Contents

Student Ambassador Update

The Stem for Life Foundation is happy to announce a recent new addition to its Student Ambassadors for the Cellular Age program, Paola Suárez Meade. Paola is currently a medical student at the Universidad Anáhuac in Mexico City in the class of 2019, specifically pursuing treatments for spinal cord injury.

Paola Suárez Meade, Universidad Anáhuac

Paola Suárez Meade

Paola explains her interest in the program by writing: "The future of mankind relies on the promising therapies of regenerative medicine. Moreover, from an economic perspective, the use of adult stem cells can be beneficial on a cost-benefit analysis by reducing the volume of ill patients, thus providing better stability to health systems. By creating awareness about the use of adult stem cells, health professionals will have the opportunity to focus on improving health by diminishing human suffering. If these therapies prove successful, we may live to be part of results we thought we could never achieve; leading cancer patients to complete recovery, delivering fully successful organ transplants, and witnessing paraplegics walking."

Paola created an engaging video on adult stem cells and the future of medicine through cell therapy, which is now available to view on www.stemforlife.org. ■

Stem for Life Releases Video:

Jim Danhakl

An Adult Stem Cell Hero

In these pages, the Stem for Life Foundation previously told the story of Jim Danhakl, a former Navy pilot and survivor of chronic inflammatory demyelinating polyneuropathy (CIDP). Jim was wheelchair-bound and rapidly deteriorating when he underwent treatment using his own adult stem cells.

Recently, Stem for Life collaborated with Jim to create a short video piece about his story, viewable now on the homepage of www.stemforlife.org. We hope you will watch the video and share it with others to show an example

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Welcome

Dr. Robin L. Smith

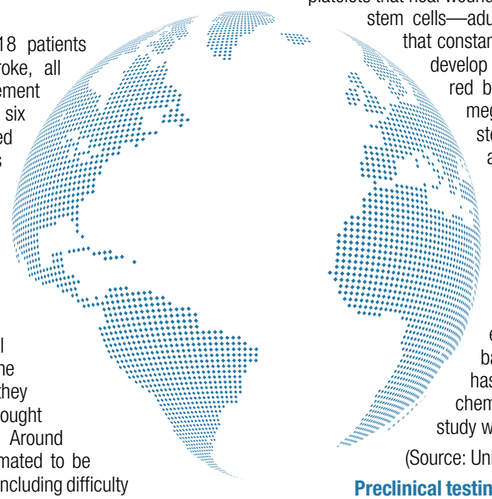
As we begin the New Year, we all think about the untapped potential the year holds for us. At the Stem for Life Foundation, we too are excited about possibilities in the year to come, in terms of continued research into life-saving cell therapies, and the opportunity for all of us to continue learning and discovering. As we consider where cell therapy will take us in the coming year, we look to individuals like Student Ambassador Paola Suárez Meade, a medical student at the Universidad Anáhuac in Mexico City who is pursuing treatments for spinal cord injury. We also revisit the story of Jim Danhakl, a survivor of chronic inflammatory demyelinating polyneuropathy (CIDP), a usually devastating autoimmune disorder, who underwent successful treatment using his own adult stem cells. Now, Jim has worked with us to create a dynamic video about his story to serve as a tool to build public awareness of adult stem cell therapies. We also hear in this issue from Dr. Tony Florino, CEO of BrainStorm Cell Therapeutics, a cell therapy development company focusing on treatments for neurodegenerative conditions. Finally, you can also take a look at our adult stem cell world news highlights for a glimpse into what scientists have discovered in the past few months.

Thank you again for your interest in our cause. ■

Dr. Robin L. Smith
President and Trustee

Adult Stem Cell World News Highlights

SFLF is pleased to share some recent developments happening around the world in the field of regenerative medicine.



In an early human study of 18 patients who had recently suffered a stroke, all 18 patients saw some improvement in weakness or paralysis within six months of having stem cells injected into their brains. Two of the patients showed significant improvement, starting to regain the ability to talk and walk the morning after their operations. The stem cells used in the study came from bone marrow donated by two people who were unrelated to the study participants. Special cells called mesenchymal stem cells were isolated from the marrow and grown in a lab, where they were treated with a gene that's thought to enhance their healing abilities. Around 7 million American adults are estimated to be living with the aftereffects of stroke, including difficulty speaking, weakness, paralysis, and trouble with thinking and memory. Findings were presented at the American Association of Neurological Surgeons annual meeting in San Francisco in April.

(Source: Health.USNews.com, 4/7/14)

New data from Harvard Stem Cell Institute scientists at Boston Children's Hospital suggest that the billions of blood cells that we produce each day are made not by blood stem cells, but rather their less-pluripotent descendants, called progenitor cells. Researchers believe that blood comes from stable populations of different long-lived progenitor cells that are responsible for giving rise to specific blood cell types, while blood stem cells likely act as essential reserves. The work, published in *Nature*, suggests that progenitor cells could potentially be just as valuable as blood stem cells for blood regeneration therapies.

(Source: Harvard Stem Cell Institute, 10/5/14)

Scientists at Stowers Institute for Medical Research have discovered that megakaryocytes (cells found in bone marrow, which are best known for producing platelets that heal wounds) directly regulate the function of murine hematopoietic stem cells—adult stem cells that form blood and immune cells and that constantly renew the body's blood supply. These cells can also develop into all types of blood cells, including white blood cells, red blood cells, and platelets. This research suggests that megakaryocytes might be used clinically to facilitate adult stem cell regeneration and to expand cultured cells for adult stem cell transplants.

(Source: MedicalPress.com, 10/14/14)

In a Phase 1 trial, researchers at the USC Davis School of Gerontology found that cycles of fasting—periods of no food for two to four days at a time over the course of six months—killed off older and damaged immune cells and generated new ones. This marks the first evidence of a natural intervention triggering stem-cell based generation of an organ or system. This discovery has potential implications for chemotherapy tolerance, as chemotherapy traditionally weakens the immune system. The study was published in the June issue of *Cell Stem Cell*.

(Source: University of Southern California, 6/5/14)

Preclinical testing done by researchers from the Icahn School of Medicine at Mount Sinai revealed that administering stem cell factor (SCF) directly into damaged heart tissue shortly after heart attacks induced the recruitment and expansion of adult cardiac stem cells to injury sites that reversed heart attack damage. The therapy also improved cardiac function, decreased heart muscle cell death, increased regeneration of heart tissue blood vessels, and reduced the formation of heart tissue scarring. About 600,000 people die of heart disease in the United States every year—that's one in every four deaths in the country, making heart disease the number one killer in America.

(Source: GenEngNews.com 11/20/14)

Researchers at Boston Children's Hospital have reprogrammed mature blood cells from mice into blood-forming hematopoietic stem cells (HSCs), using eight genetic switches called transcription factors. The reprogrammed cells, called induced HSCs (iHSCs), have the functional hallmarks of HSCs, are able to self-renew like HSCs,

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An Adult Stem Cell Hero - Continued from page 1

of the incredible progress being made in medicine and how adult stem cell therapies may revolutionize healthcare as we know it.

For his bravery in the face of illness and his commitment to bettering the lives of others by raising awareness of adult stem cell therapies, the Stem for Life Foundation and Pontifical Council for Culture recently presented Jim with the Pontifical Hero Award. Thank you, Jim, for your efforts in support of our cause. ■

News Highlights - Continued from page 2

and can give rise to all of the cellular components of the blood like HSCs. The findings mark a significant step towards the ability to produce HSCs suitable for hematopoietic stem cell transplantation (HSCT) from other cell types, in particular more mature or differentiated cells. Only about one in every 20,000 cells in the bone marrow are HSCs, so the ability to generate induced HSCs from a patient's other cells could have an impact on transplant medicine and diseases of blood development. The findings were reported in the journal *Cell*.

(Source: Children's Hospital Boston, 4/24/14) ■

Five Questions with Tony Florino

Tony Florino, MD, PhD, CEO, BrainStorm Cell Therapeutics

Tony Florino, MD, PhD.

Q: What is the mission and goal of BrainStorm Cell Therapeutics?

A: BrainStorm's mission is to develop stem cell-based therapeutics for the treatment of neurodegenerative diseases. Our platform technology, called NurOwn, consists of autologous mesenchymal stem cells (MSC) that have been induced, under our proprietary culture conditions, to secrete a variety of neurotrophic factors, which are growth factors known to promote neuronal survival. Within this mission we have several goals – the first is completion of the ALS clinical development program, which is currently in Phase 2 and could be in a pivotal study in 2017. (Amyotrophic lateral sclerosis [ALS] often referred to as *Lou Gehrig's Disease*, is a progressive neurodegenerative disease that affects nerve cells in the brain and the spinal cord.) A second important goal for BrainStorm is to broaden the pipeline by moving additional indications for NurOwn into the clinic, while embarking on preclinical development in new areas.

Q: Can you briefly discuss NurOwn and its mechanism of action?

A: As mentioned, NurOwn is derived from mesenchymal stem cells. We obtain these MSCs from a bone marrow aspiration, followed by separation of the MSCs and expansion. After about three weeks, the MSCs are placed under proprietary culture conditions, which induce the cells to produce several neurotrophic factors. We believe that NurOwn cells, acting as a biological drug delivery system, can deliver neurotrophic factors to stressed, injured, or dying neurons, which could extend the survival of those cells, and consequently, halt or slow disease progression. In addition, MSCs have immunomodulatory effects that may play an additional role in the activity of NurOwn.

Q: What did the Phase 2a trial of NurOwn reveal?

A: We're very gratified with the results of this study. The safety and tolerability profile was good, with the vast majority of adverse events being low-grade and resolving within two days of administration. We saw that almost all of the subjects in the study experienced a benefit from NurOwn, defined as a slowing of disease progression (assessed by the Amyotrophic Lateral Sclerosis Functional Rating Scale [ALSFRS] or forced expiratory vital capacity [FVC]) for the three months after treatment compared to the three months prior to treatment. When we look at how those scores changed over time, we saw a very impressive reduction in the rate of disease progression for both ALSFRS and FVC. For ALSFRS, the rate of disease progression was reduced by 57% going out six months after treatment, and for FVC, disease progression was slowed by 67%. The magnitude of benefit we observed in this study is large and clinically meaningful, and if we see a similar result in a larger study or studies, we will be very well-positioned to seek regulatory approval.

Q: What do you expect to test, and to learn, in further NurOwn trials? What challenges will you be facing?

A: The next clinical trial that we are currently planning is a multi-dose study. All of our trials so far, including the study we are enrolling in the US, have featured a single administration of NurOwn. However, we expect that for progressive diseases like ALS, ongoing treatment would be necessary. A multidose study will allow us to test NurOwn cells made from cryopreserved MSCs, and assess the safety and efficacy of (most likely) three doses administered at two- or three-month intervals. This study does not present any special challenges, but we will be focused on flawlessly integrating the use of cryopreserved cells into the manufacturing process.

Q: Where do you see the greatest potential for adult stem cells in neurodegenerative diseases in the next 5-10 years?

A: I would be remiss if I didn't take this opportunity to mention how excited I am about the breadth of possible applications for NurOwn across a wide variety of neurodegenerative diseases. I am looking forward to moving multiple projects into preclinical development and from those, identifying new clinical indications over the next several years. Moving beyond BrainStorm, I think there is good chance we will see further advances in the use of biomaterials and scaffolds in combination with adult stem cells for treating peripheral nerve and spinal cord injuries. And finally, the study of stem cells in CNS diseases would be greatly facilitated by the development of catheters or other techniques that would allow administration of multiple doses of cells directly into the brain. ■