Stem for Life Foundation
www.stemforlife.org
October 2013
Life Lines

Contents

A CIDP Survival Story

Mr. James Danhakl, Stem Cell Patient

I will never forget the moment that my life changed—June 30, 2009. I had slept in, and was looking forward to a leisurely stroll. As I lowered my feet down to the carpet, I felt an odd tingling sensation in the balls of my feet, almost like a low voltage electrical charge.

I spent much of the past 20 years as a Navy fighter pilot and had always taken care of myself. That summer I had commenced an exercise program that involved weight training, aerobics and power walking 50-60 miles per week. That day I lifted weights for two hours, rode the bike, then power walked a 26 mile marathon. When I hit the bed that night, I felt a fatigue deeper than anything I had ever experienced. I had just "overdone it." I figured it would all be better with a couple of days rest. I was wrong.

Jim's first day at Northwestern Memorial Hospital

Over the course of the next two weeks the tingling feeling changed to numbness and began to creep up my feet. I felt a loss of coordination in my knees. I was losing physical strength. It wasn't until the third week, when I began to feel the tingling in my fingertips that I really became concerned. I realized that whatever was happening was systemic and not just a training injury. I went to my family doctor who referred me to the local neurologist. It took two weeks before I could get an appointment. The neurologist diagnosed me with Guillane-Barre Syndrome (GBS), an acute autoimmune disease that demyelinates the peripheral nervous system. At least they knew what was wrong with me. It had a name. We could treat it.

I was admitted to the hospital and spent the next five days receiving daily infusions of Intravenous Immuno

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Welcome

Dr. Robin L. Smith

I am pleased to present this October issue of *Life Lines*, with updates about our work at the Stem for Life Foundation (SFLF) and important developments occurring in the field of adult stem cell therapies. I hope these articles encourage you to join us in our mission of raising global awareness about the benefits of stem cell therapies and regenerative medicine.

In this issue, read a special story of courage and determination, shared with us by stem cell therapy patient Jim Danhakl. Jim experienced a crippling and life threatening brush with chronic inflammatory demyelinating polyneuropathy (CIDP). He participated in a stem cell clinical trial at Northwestern University, which in his words "saved his life." Jim's story reaffirms our hope that regenerative medicine will transform the lives of those with severe medical conditions.

Our special education segment brings together three leaders in the field of adult stem cell transplants, Dr. Amitabha Mazumder, Dr. Stephen Nimer and Dr. Edwin Horwitz, to present us with an overview of the field and bring us up to speed on some of the interesting developments.

We also have shared some of the successes of the inaugural class of our Student Ambassadors for the Cellular Age Program. These students are making strides to spread awareness of stem cell therapies and regenerative medicine at their universities and around the globe.

In this issue we also are introducing a new toolbox on our website to provide you with the information you need to help advance our mission in your own communities. We have also highlighted some exciting examples of scientific developments in the latest Adult Stem Cell News Highlights.

You can learn more about the Stem for Life Foundation and our activities at www.stemforlife.org. Thank you for being a part of our cause. ■

Dr. Robin L. Smith
President and Trustee

Adult Stem Cell News Highlights

SFLF is pleased to share some exciting recent developments in the field of regenerative medicine:

- Researchers in Japan's Riken Center for Developmental Biology and the Institute of Biomedical Research and Innovation Hospital in Kobe will use stem cells to attempt to treat age-related macular degeneration, a form of blindness. *(Source: BBC News, 7/19/13)*
- Researchers at UCLA's Jonsson Comprehensive Cancer Center announced that they have successfully combined cellular therapy (using T cells) and gene therapy in a mouse-model system to develop a viable treatment strategy for breast cancer that has spread to a patient's brain. *(Source: UCLA press release, 8/02/13)*
- Texas Governor Rick Perry announced a $3 million investment through the Texas Emerging Technology Fund to create the Center for Cell and Organ Biotechnology in collaboration with the Texas Heart Institute and Texas A&M University College of Veterinary Medicine and Biomedical Sciences. The new center will allow students to "benefit from participating in the use of advanced stem cell technologies to advance the research of cardiovascular science, personalized medicine, organ replacement, regeneration and repair, and more." *(Source: BioNews Texas, 9/16/13)*
- In a study published Sept. 18 in *Nature*, Israeli researchers reported that they identified the key molecule that stops adult cells from transforming into so-called induced pluripotent stem (iPS) cells. Those stem cells are similar to the primitive cells found in embryos, and have the potential to generate any type of body tissue, and could potentially be used to treat a variety of chronic diseases. *(Source: HealthDay News, 9/18/13)*
- The McGowan Institute for Regenerative Medicine, a program of The University of Pittsburgh School of Medicine and University of Pittsburgh Medical Center, will co-direct a $75 million, five-year project nationwide to continue developing tissue-engineering techniques to treat battlefield injuries. The funds will be used "for ongoing research to develop ways to heal wounds and burns; develop a computer-controlled spray gun to spread adult stem cells atop burns to advance recovery; use tissue matrices to regenerate skin and other tissue to heal wounds; improve a drug to help stem cells penetrate wounds and prevent scarring; and continue research in regenerating ligaments, tendons and joints." *(Source: Pittsburg Post Gazette.com, 9/28/13).* ■

How You Can Help

Providing You with Tools to Help Spread the Word

The Stem for Life Foundation is happy to announce that we now provide online resources to empower our supporters to directly participate in our mission to raise public awareness of adult stem cell therapies and support adult stem cell research and development. These tools provide an opportunity for supporters, who share our passion for adult stem cells, to spread the word among family, friends, and their communities.

Our fundraising toolbox, located at www.stemforlife.org/spreadtheword, provides tools and resources that can be used to educate yourself and your contacts, raise awareness, and engage in local media outreach.

Resources include:

- An exciting adult stem cell overview video presented by Dr. Max Gomez, WCBS Medical Correspondent.
- An informative SFLF brochure with information on adult stem cells, as well as the SFLF mission statement and goals.
- An archive of our past *Life Lines* newsletters presenting an overview of events and issues happening in the field of adult stem cells.
- A digest of adult stem cell and SFLF news aggregated from reputable sources.

Tips on Making a Presentation on Adult Stem Cells

SFLF is happy to provide you with slides and video that can become the basis for your presentation.

Just keep these considerations in mind:

- **Know your audience** and provide the necessary background based on their level of understanding
- **Present a balance** of powerful patient profiles and validated scientific statistics
- **Keep it short** (20 mins or less) and leave time for questions
- **Refer them to stemforlife.org** and other online resources so audience members can learn more

The SFLF team is available to discuss your ideas and assist you with generating interest in our cause; making an adult stem cell PowerPoint presentation; creating a fundraising event; or writing letters to community members, local schools, government officials or neighbors to increase awareness and ask for support. You can contact us at info@stemforlife.org.

With your help, more people will understand the potential that adult stem cell therapy research and development offer in the fight against chronic illness. Help us spread the word! ■

Student Ambassador Updates

The Stem for Life Foundation is proud to share some updates about the important work being done around the globe by our **Student Ambassadors for the Cellular Age.** Our Student Ambassador program aims to inspire the next generation about the potential of and advances in adult stem cell science and the role cellular therapy will play in medicine and in alleviating human suffering. Many of our Student Ambassadors participated in the Second International Vatican Adult Stem Cell Conference and have since returned to their schools and universities where they are raising awareness about regenerative medicine among their peers. For more information about becoming a Student Ambassador please email studentambassadorprogram@stemforlife.org.

Dr. Joseph Bernard, Jr.

University of Notre Dame, Haiti

Joseph delivered a well-received presentation during the annual scientific conference, *Journées Scientifiques*, at University of Notre Dame, Haiti. He presented to a group of physicians, nurses and students on the topic of regenerative medicine entitled: "Regenerative Medicine: Current Perspectives and Opportunities for Haiti" *(La médicine regenerative: Persepectives actuelles et opportunités pour Haiti).* Joseph practices medicine in the area of infectious diseases.

Humberto Mestre Payne

Universidad Anáhuac, Mexico

Humberto is helping to organize a national conference at the Universidad Anáhuac in celebration of the 50th anniversary of the University. In partnership with the National Transplant Center and Mexican Academy of Medicine, the conference will be held in Mexico City in March

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Special Education Segment

General Principles of Adult Stem Cell Transplants

Dr. Amitabha Mazumder

Professor, *New York University School of Medicine*


Dr. Amitabha Mazumder

Adult stem cell transplants (also referred to as bone marrow transplants) have been used for over 40 years in successfully treating cancers such as leukemia, multiple myeloma and lymphomas, and research has now opened the doors to regenerative and reparative therapeutics.

There are three different categories of patients who would benefit from stem cell transplants:

- Those patients who don't have stem cells, or if a patient's bone marrow stops working and doesn't produce enough healthy stem cells. Example - patients with aplastic anemia, a severe blood disease, where the myeloid stem cells are absent.
- Patients who suffer from genetic diseases where the stem cells are present, but are considered "defective," including sickle cell disease and certain autoimmune disorders.
- Cancer patients. When you are using the patient's own cells in an autologous setting (using your own cells), you are replacing blood-forming cells destroyed by cancer treatments. The goal of a stem cell transplant is to help the bone marrow recover and continue to make healthy blood cells.

Perhaps the area where stem cell transplants have made the most headway is for cancer patients. Stem cell transplants have cured thousands of patients with what were thought to be incurable cancers. These patients would normally be dying and are essentially being rescued by autologous stem cell transplants.

As the results of the research become more definitive, we are starting to view autologous stem cell transplants as a way to reset the clock, for example, in the immune system. We are continuing to build our research efforts on these earlier successes. In an allogeneic transplant (using somebody else's cells), donor cells are intended to provide the patient with new blood cells and a new immune system. The donor cells are obtained from a Human Leukocyte Antigens (HLA) matched individual, and may be obtained from cord blood; a related donor who is a match (based on HLA, the genetic markers of the immune system); or from unrelated donors.

An exciting part of an allogeneic stem cell transplant is that we can now understand that the cells that we're giving to the patient are the cells that are actually doing the work of destroying the cancer cells. So we don't have to blast the patient with high doses of chemotherapy or radiotherapy. These cells are actually going in and they're creating what we call a graft vs. leukemia or graft vs. tumor response. This enables us to be able to do the procedure in an outpatient setting with very low doses of chemo-radiotherapy. We can infuse these cells in and let them do their work. We can do the procedure in 70-75 year olds; we can do it in patients who have renal, heart or lung dysfunction. And this process has markedly enhanced the cure rate of a lot of patients. ■

Important Developments in Stem Cell Transplants

Dr. Stephen Nimer

Director, *Sylvester Comprehensive Cancer Center, Univ. of Miami*


Dr. Stephen Nimer

This year alone, about 19,000 stem cell transplants will be performed for cancer and related diseases in the United States. Over the last 30 years, the field of stem cell transplantation for cancer has provided not only hope, but cures on a consistent basis for many types of patients.

People often ask about the difference between current types of stem cell treatments, known as *non-myeloablative transplants,* and the *conventional* version of transplants. In conventional allogeneic transplants a patient is treated with preparative chemotherapy and/or radiation to kill as many cancer cells as possible and to suppress the immune system of the

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patient to allow the donor cells to grow. A non-myeloablative transplant aims to suppress the immune system just enough to allow engraftment of the donor cells to attack the cancer.

The follow-up question is then, with a non-myeloablative transplant, why don't these stem cells attack the host, given that the host still has an immune system? What we understand now is that there is a battle that goes on between the immune cells of the host and the donor. We can create a very gentle, yet effective suppression of the immune system as long as we infuse a sufficient number of new immune cells.

Patients often wonder as to what type of donor they may need for a successful stem cell transplant. With an organ transplant, patients are given lifelong immune suppressive medications, thus the donor need not be related to the patient. However, when treating cancer, we do not want to give immunosuppressive therapy, and therefore you hope to find a donor who is closely related, but not necessarily identical to the patient. Thus, a brother or sister who is HLA matched is actually better, because the immune system that matures in the patient will have novel abilities to recognize and control the patient's cancer.

Another amazing development in the past few years has been the use of cord blood stem cells for transplants. These stem cells are nearly the same as those that are in your bone marrow, but they are found in the cord blood at the time an infant is born. These cells are more immature than the cells that you get from bone marrow, especially in terms of their immune function, so that matching the donor with the patient is much easier. We refer to things like a 10/10 match when we take cells from an adult and transplant them. For cord blood transplants, we only need about 2/3 as strict a match as we need for adults. What this has done, in particular for minorities, is opened up the ability of curative therapies to be applied throughout the world. But the negative side of using cord blood in a transplant is that it often takes 12 to 18 months for the immune system to come in quite robustly, leaving the patient at risk for infections for a longer period of time. A lot of the research now is to identify how to strengthen the immune system of cord blood transplant recipients once the process has started. The results are looking better, especially for pediatric patients. ■

Use of Mesenchymal Cells in Stem Cell Transplants

Dr. Edwin Horwitz

Professor, *The Children's Hospital of Philadelphia and UPenn*


Dr. Edwin Horwitz

I focus my research almost exclusively on the transplantation of marrow cells and mesenchymal stem cells (MSCs) to treat cancer and genetic disorders of childhood. When we do stem cell transplants using MSCs, we're not doing any pre-conditioning or depletion of the immune system. First of all, part of the role of a conditioning regimen is to get rid of some of the patient's hematopoietic stem cells (blood making cells) and allow new ones to take over. The MSCs that we infuse do not engraft in any specific place. So there's no competition and therefore, no need to get rid of anything. Secondly, the other reason to give a conditioning regimen is to suppress the patient's immune system or perhaps even get rid of their immune system so that there's no rejection of the donor's stem cells coming into the patient. Since MSCs do not seem to stimulate an immune response or rejection response in the patient, there's no need to suppress the immune system.

MSCs go into the body, go wherever they end up, and secrete their *magic* ingredients and then they circulate in the blood. We have observed that MSCs uniformly seem to migrate to sites of injury, consistent with the concept that MSCs promote the healing process by stimulating a person's own regenerating mechanisms to heal the tissue.

After we grow the cells and infuse them into the patient, we find that sometimes we get extraordinary responses. Sometimes it's good, but not extraordinary. What if we could engineer the cells so as to yield an extraordinary response each and every time? That would be a huge step forward, but to do that we need to understand better how they're working. That's why it's important to isolate the MSCs and investigate their secreted molecules, the magic ingredients, which is one of the specific areas that we are researching today.

Perhaps someday we may be able to isolate what the MSCs are secreting and treat patients with this substance (as opposed to treating with MSCs). I'm not sure it will work as it can be difficult to reproduce everything exactly as the MSC does it. But if we understand what that molecule is and how it works, we could potentially select cells, or even engineer cells that do a better job at healing the tissues, to make a better cell therapy with better outcomes for patients. ■

To read the full text of these articles, please visit stemforlife.org/blog.

A CIDP Survival Story

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Jim walking his daughter down the aisle

globin (IV-IG). While in the hospital I researched GBS and I realized that my disease profile was different. Where GBS strikes within hours and days, hitting its maximum within a week or two, my symptoms had been going on for over a month and were only increasing. I read that if GBS symptoms persist, the disease can sometimes take on its chronic form – chronic inflammatory demyelinating polyneuropathy (CIDP). CIDP is more rare, and while usually not fatal, is also a permanent disease, much like multiple sclerosis (MS) where patients go up and down, but over time mostly down.

After I left the hospital, I continued to decline. By the two-month point I was having difficulty just walking around the house. I could no longer drive. I was losing sensation in my arms hands and torso. My world was becoming small.

I cannot begin to describe what it is like to lose so much so fast. So much of our daily world is experienced by movement and by our senses. It felt like I was being robbed relentlessly of my very being. I could not sleep this away, pray it away, eat it away or "positive attitude" it away. I had to find help.

I began to see "big league" doctors at major teaching hospitals. But they had no answers. I found a support blog online for GBS and CIDP patients. Reading these personal accounts was devastating. Most people over a period of years decline to a point of severe disability. Some die from complications. There are no cures or even treatments that change the long-term prognosis.

I noticed a thread titled "Stem Cell Transplant for CIDP". The thread chronicled the journey of Alice, a CIDP sufferer, and her steadfast determination to undergo a new and possibly game changing experimental treatment. We spoke, and we compared our disease progressions, which were remarkably similar. We also shared similar frustrations with the medical world and how little conventional medicine had to offer. She was undergoing a procedure for autologous stem cell transplantation, a process developed at Chicago's Northwestern University by Dr. Richard Burt. In simple terms this process "reboots" the immune system much like rebooting a computer after it becomes too slow.

From that moment on, I had a singular focus to get to Northwestern and have this procedure. By this point I was four months into the disease, and had tried steroids, IV-IG and chemotherapy, none of which had any impact or slowed the disease progression at all. My progression was in fact unusually fast. I was hitting disability wickets in weeks that most people take years to develop. This decline looked like the rarest and most malignant form of the disease. My loss of physical control was becoming pronounced. I spent most of my days in bed. I had a walker, but several times I would pass out from just standing.

But my spirits were up. Despite my worsening condition, I now had hope, and I cannot tell you how important that was to me. I felt a sense of total optimism about the prospect of a stem cell transplant. In simple terms, the procedure requires first harvesting a patient's adult stem cells from his bone marrow and then killing the existing immune system using chemotherapy. The stem cells are then reintroduced into the body which then grow into an immune system that is genetically identical to the one that you were born with, but with none of the memory and bad habits it picked up along the way. Dr. Burt developed the protocol initially for MS patients, but found the procedure effective against many other autoimmune diseases such as lupus, Crohn's, systemic sclerosis and thankfully, CIDP.

I was admitted into the clinical trial a month later. From the moment I arrived at Northwestern, I knew I had made the right choice. I won't go through all the details of the stem cell transplant. But I was taking my first steps within a couple of weeks. Within six months I had 90% of my function back. Within a year I had all my function back and today, three and half years out from the transplant I am as strong as ever. There have been challenges along the way. I did have pain that lingered for some time after the procedure but most of that has thankfully resolved.

I still cannot run and cannot walk more than a few miles without taking a break. But I live a full and complete life. And from where I came from this is a miracle. There were some psychological challenges. We are all imbued with a confidence in our physicality. It enables us to function and take risks in life. That confidence took a major hit, but over time has largely returned. While I will never forget what happened, I consider myself cured, and that feeling is like being innocent all over again. The best part about not forgetting is the enormous gratitude you get as a survivor. Every morning I put my feet on the ground and stand, I am filled with gratitude, for just being able to do that. I owe my life to Dr. Burt, my wonderful friend Alice who guided me through it, and my family and close friends whose love and support literally saved my life. ■

To read Jim's full article, please visit stemforlife.org/blog.

Student Ambassador Updates

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2014. One of the themes will be regenerative medicine, with a special emphasis on cell-based and organ-based transplants. Humberto is a 2014 candidate for a Bachelor of Medicine and Surgery at the Universidad Anáhuac.

Angela Chlebowski

Fairfield University, Connecticut

Angela and her experience at the second Adult Stem Cell Conference were featured in an article for *The Pulse*, the publication of the School of Nursing at Fairfield University. Angela will present about her areas of interest in regenerative medicine, as well as her experience at the Vatican Conference at a nursing conference later this year. Angela is a graduate student at Fairfield University where she studies Integrated Healthcare Leadership.

Rebecca Marton and Kristin Springer

University of Notre Dame, Indiana

In the spring, Notre Dame seniors Rebecca Marton and Kristin Springer and their experiences at the Vatican Conference were featured in an article for *The Observer*, their campus newspaper. They are also planning to write an article for *Scientia*, their campus undergraduate science journal, as well as to give a presentation of what they learned in Rome at both a *Scientia* meeting and a biology club meeting.

Rebecca and Kristin are both majoring in Biology. Rebecca studies retinal regeneration in zebrafish and will be soon be presenting her findings at the Midwest Society for Developmental Biology meeting at Washington University in St. Louis, Missouri. Kristin is performing her undergraduate research on kidney regeneration in the zebrafish, and presented her work this summer at the Midwest Zebrafish meeting in Milwaukee, Wisconsin.

Raquel Diaz

Pontificia Universidad Javeriana, Colombia

Raquel has been teaching a lecture course on the uses of the human body in biotechnological research from a bioethical perspective, which is also the topic of her Masters' dissertation. Although the main topic of her research is genetic transformation and liberal eugenics, she has incorporated discussions of adult stem cell research into some of her focus groups, as well as her bioethics and medical ethics lectures. Raquel is a graduate student at Pontificia Universidad Javeriana, and a faculty member in the Department of Political Science at La Universidad Icesi in Colombia.

Samuel Velásquez Serrrano

Pontifica Universitá Gregoriana, Italy

Samuel wrote an editorial response to *Nature's International Weekly Journal of Science's* editorial "Smoke and Mirrors". His response promoted the virtues of the Vatican Conference and its focus on ethical and responsible science. In May, Samuel gave a presentation about the Vatican Conference to all Ph.D students and the Faculty of Moral Theology. In July, Samuel made a presentation about adult stem cells and regenerative medicine to a group of Seminarians (youth who are preparing for the priesthood) in the Archdiocese of San Juan in Puerto Rico. Samuel is a graduate student at Universitá Gregoriana where he studies Moral Theology and Bioethics.

Nikhil S. Bardeskar

St. Xavier's College-Autonomous, India

Nikhil is scheduled to make a presentation about adult stem cell research to some convent schools in Mumbai this October. He has also shared his experience from the Vatican Conference through an active Facebook group he started "Adult Stem Cell Awareness Group" where he regularly posts articles and other information to spread awareness about adult stem cells. Nikhil is a candidate for a Masters of Science in Biotechnology at St. Xavier's College-Autonomous in Mumbai. ■

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:

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To learn more about becoming a Student Ambassador:

Email studentambassadorprogram@stemforlife.org

The Stem for Life Foundation's mission is to increase awareness of, and access to, current and potential stem cells therapies, and to support adult stem cell research.

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