Research using adult stem cells continues to yield successful treatments for many human diseases and injuries. In this update we highlight some of those treatment successes from the last six months. This update follows on our previous releases of adult stem cell success stories from the first half of 2008, as well as from 2007 and 2006,¹ and our pamphlet with stories and pictures of patients successfully treated with adult stem cells.²

Autoimmune diseases

First Bari Martz's fingers turned blue. Then she started gasping for breath and her joints stiffened so that she could not open her hands. Doctors diagnosed scleroderma, part of a family of diseases which attack the immune system of a patient's own body. They treated it by using stem cells from her blood.

Doctors here and in Europe are aiming to reset the immune systems of patients with severe scleroderma. If they are successful, it will cast new light on numerous autoimmune diseases, from lupus to multiple sclerosis. Autoimmune diseases are among medicine's most frustrating mysteries. Arguably most mysterious is scleroderma, where the immune system somehow mistakenly attacks connective tissues that support the skin and

² Adult Stem Cell Treatments-9 Faces of Success, http://www.frc.org/get.cfm?i=BC06I01
internal organs. The disease thickens skin, stiffens joints, and destroys blood vessels, sometimes leading to death through kidney and lung failure.

The theory is that someone genetically predisposed to certain autoimmune diseases stays healthy until something in the environment triggers misfiring immunity. Stem cells, however, should not be diseased, explains Dr. Keith Sullivan of Duke University, who is leading the largest study to date of the transplants, called the SCOT trial.3

"There was not a choice," Martz, now 49, of Parkland, Florida, says of volunteering for the SCOT trial. She was losing about 10 percent of her lung function a month, and feared she had less than a year left to live when she underwent her January 2007 transplant. "If I died from it, well, at least I went out fighting." For now, "I'm great," she says. Her lung function jumped and is still improving; she can flex her hands again and can even climb stairs, if slowly: "I'm continuing to get better." 4

Brain Injury

New nerve cells, produced naturally by adult neural stem cells present in the brain, appear to be essential for learning and memory. The old idea that brain cells are not renewed was debunked in the 1990’s, when researchers showed that the adult brain continues to make new neurons, a process termed "neurogenesis," throughout life. Now, researchers at Kyoto University in Japan have shown in mice that new brain cells are necessary for learning and for memory. The new research, published in Nature Neuroscience, indicates that neural stem cells in the adult brain continue to produce new brain cells that are important for memory and learning.

The new study supports work published earlier this year showing that new brain cells can affect learning and memory. Last year, research showed that transplanting adult neural stem cells into brain-injured mice could restore some memory. In March 2008, another study showed that injecting human umbilical cord blood stem cells into the brains of aging animals boosted neurogenesis. Another recent study also suggested that stimulating specific molecules in the brain could reactivate adult neural stem cells. Exercise has also been shown to stimulate neurogenesis in the brain.5

An Auckland twin who was **brain-damaged** at birth has become the first New Zealander to undergo experimental treatment in the United States using her own umbilical cord blood. Three months ago, **Maia Friedlander**, 4, was locked in her own world. Despite six hours of therapy a day for three years, Maia—who was born six weeks premature—struggled to talk, walk properly, or even chew her food without choking. Her twin sister, Ariel, achieved all her developmental milestones about six months early, but Maia did not learn to crawl until she was three. Her father, Daniel, said: “Our lives revolved around her therapy regime but we could not see much improvement.”

The breakthrough came in February, when they met American mother Mary Schneider, whose son, Ryan, was the first to undergo cord blood transfusion for his brain injury at Duke University, North Carolina. Five years later, he is developmentally normal. More than 50 other children with brain injuries have been treated through Duke's reinfusion program. In August, Maia and her mother, Jillian, traveled to the U.S., where she received a two-hour infusion of her own cord blood stored by her parents at birth. Within days her concentration and coordination improved. Maia now goes to kindergarten five days a week. "She's like a different child—talking, hugging us, playing . . . She's had a second chance at life and we can now have the family life we'd always dreamed of."  

**Stroke**

Doctors have used a revolutionary stem cell treatment to restore the power of speech for a **stroke victim**. **Walter Bast** also regained the use of his right arm after the operation to place a “teabag” of drug-producing adult stem cells in his brain. Speaking a week after the operation—the first of its kind in the world—he said: “I feel like a lucky guy.” If further trials confirm the value of the treatment, it could be on the market in as little as five years, providing fresh hope for the 45,000 Britons each year who suffer a hemorrhagic stroke caused by the bursting of a blood vessel in the brain. Currently, the only treatment option is surgery, which has a variable success rate. Half will die within a month and just one in 20 patients will recover to the extent of Mr. Bast, a 49-year-old mechanic. British experts described the operation as “very promising.”

The CellBeads treatment is the brainchild of scientists at the British medical technology firm Biocompatibles International, based in Farnham, Surrey, U.K. At its center is a teabag-like sachet filled with tiny capsules, each containing approximately a million stem cells. The stem cells, taken from bone marrow,

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have been genetically engineered to produce a drug that protects brain cells from
dying. This allows the cells to rejuvenate and repair the damage done by the
stroke. The stem cells are encapsulated in beads to hide them from the immune
system and ensure they are not rejected by the body.7

Cerebral Palsy

When Chloe Levine was 9-months-old, her parents noticed she could not hold
her bottle with her right hand. That was not her only developmental setback.
Chloe, of Pinetop, Arizona, was unable to raise both hands above her head and
could not crawl. At 12 months, a CAT scan showed a portion of the left side of
Chloe’s brain had not developed and contained fluid. Chloe’s parents, Ryan and
Jenny Levine, took her to a neurologist who diagnosed the toddler with right-
side hemiplegic cerebral palsy. “The cerebral palsy had only affected the right
side of her body,” Jenny Levine said. “The neurologist told us we were looking at
17-18 years of therapy.” That was when the Levines heard about an experimental
procedure at Duke University in North Carolina, where children with cerebral
palsy were infused with their own cord blood stem cells in an effort to heal and
repair damaged brain tissue.

The Levines remembered they had banked Chole’s cord blood when she was
born. “It was a miracle,” Dr. Manny Alvarez said on FOX & Friends. “I
congratulate you for banking her cord blood. Stem cells are a new field of
medicine and they certainly can rejuvenate the tissue.” Two months ago, Chloe,
2, received an infusion of her own stem cells and her progress is remarkable, said
her father, Ryan. “Her therapist said she’s made a 50 percent recovery,” he said.
“She can walk, run, and do sign language with her right hand.”8

Spinal Cord Injury

The Australian team at the National Centre for Adult Stem Cell Research,
Griffith University, continues to produce exciting results. The latest report
published in the journal Brain gives the results of a 3-year clinical trial, using
olfactory ensheathing cells (specialized adult cells that surround nerves) from the
patients' own noses, transplanted into the damaged spinal cord. The initial one
year follow-up has shown no adverse effects from the transplant.

7 Macrae, Fiona, “The teabag: Stem cells in a pack help stroke victim to talk again,” Mail on Sunday
(December 2, 2008). Accessed at: http://www.mailonsunday.co.uk/health/article-1091445/The-miracle-
tebag-Stem-cells-pack-help-stroke-victim-talk-again.html
http://www.foxnews.com/story/0,2933,392061,00.html
This was a highly controlled trial, with matched control and transplant patients, followed for 3 years. Patients were chosen who might be considered "chronic"—at least 2 years after their spinal cord injury—to control for any spontaneous recovery. The trial was designed to show the safety of the transplant. The transplant was safe by all measures, and one transplanted patient showed improvement over 3 segments in light touch and pin-prick sensitivity. The cells were shown to be quite safe, to take well in the patients, and to safely improve function.9

Cancer

Susan Fister nearly died four years ago—but a cord blood transplant saved her life. It all started when the Medina woman fell at work and developed a bruise that would not heal. She went to have it checked out and discovered she had leukemia. She had two bone marrow transplants, but both failed. Her doctor suggested trying something different—an adult stem cell transplant from umbilical cord blood. In the weeks after the cord blood transplant, she became stronger and her body did not reject the new cells. She has not been back in the hospital since November of 2004.

Susan has noticed that her new immune system has had other benefits as well. When she fell off a table and tore a ligament in her knee, her doctor was shocked by how quickly she recovered. And she reports her skin heals faster too. Mary Laughlin, her transplant physician at University Hospitals Ireland Cancer Center, says it is not surprising the stem cells have affected more than her leukemia. These cells not only cure the leukemia or life-threatening blood disorder, they also participate in the repair of all the organs in the body.10

At Kansas State University researchers are working on a method of delivering cancer drugs that promises to be more efficient and reduce side effects. Researchers are studying how stem cells can be used to deliver anti-cancer drugs directly to breast cancer cells via nanoparticles. The researchers are using adult stem cells isolated from Wharton's jelly, the substance that cushions blood vessels in the umbilical cord. These types of stem cells can be harvested non-invasively and therefore are not controversial. "Billions and billions of these cells are disposed of every day," Professor Deryl Troyer said, "We think these cells have a lot of advantages, including their ability to be harvested in large numbers

very rapidly." The stem cells tend to travel to tumors and other pathological lesions. The researchers are using these adult stem cells as delivery systems by loading the cells with nanoparticles that contain anti-cancer drugs.11

Researchers have discovered adult stem cells in the prostates of mice, and have grown complete prostates from a single prostate adult stem cell. The team at California-based Genentech Inc. said human beings have similar stem cells in their prostates, although so far they have not grown human prostate glands from the cells. Other researchers said that the research might lead to better ways to fight prostate cancer and the common enlargement of the prostate that comes with age. "A total of 14 prostates were generated from 97 single cell transplants," the Genentech team wrote in their report, published in the journal Nature. "This is, to our knowledge, the first report to demonstrate prostate generation from a single adult stem cell." 12

Bill Dubois of Phoenix is an Army veteran: his sister, Sidney Wallace, is a former Marine. So it was appropriate that around Veteran’s Day of 2008, Dubois had been cancer-free for a little more than a year thanks to the adult stem cells his sister donated. Dubois had been diagnosed with non-Hodgkin’s lymphoma five years earlier. Chemotherapy had suppressed the lymphoma for several years, but it returned. In the meantime, advances in treatment had shown that an adult stem cell transplant could give him a better chance of recovery. His sister was a perfect match for the transplant, and was happy to donate adult stem cells for her brother. A year later and free from lymphoma, Dubois says, “It makes you want to enjoy every day.”13

Immune Deficiency

Nine-month-old Granton Bayless has a rare condition called severe combined immunodeficiency that prevents his body from producing enough T cells to fight off disease. Admitted to Children’s Mercy Hospital on March 28, Granton battled pneumonia and respiratory syncytial virus so severe that holes developed in his fragile lungs. Medical staff placed him on a ventilator because he could not breathe on his own. They inserted tubes into his chest to suction air leaking from his lungs. They administered medicine that paralyzed him so equipment would not hurt him if he moved and so he would not burn energy or oxygen.

Eventually Granton's parents made the critical decision to go ahead with an umbilical cord-blood transplant in which cells would slowly attack the virus. Umbilical-cord-blood cells are taken from a baby's umbilical cord and placenta. Granton continues to improve: a recent blood test on him showed that 86 percent of his white cells were donor cells. A recent test showed Granton had produced "natural killer cells," which play a major role in fighting infections. Now Granton plays with everything in his reach.14

**Heart Tissue Regeneration**

Nearly five million people in the U.S. suffer from congestive heart failure. For some, a heart bypass procedure will work. But now, growing your own bypass, with your own adult stem cells, may be a possibility. That is how Lieutenant Ronnie Smallwood sees it. Smallwood suffered from congestive heart failure. He was treated by putting some of his own adult stem cells into parts of his heart muscle. Smallwood is now feeling much better, and ready to go back to fishing in his off hours. He was treated by Dr. Emerson Perin of the Texas Heart Institute, who has treated a number of heart patients with their own adult stem cells. “What we are doing with the stem cells is hopefully creating better blood flow to areas of the heart that do not get good blood flow,” Dr. Perin says.15

Adult stem cells collected at birth from the umbilical cord may help doctors fashion new heart valves for children born with heart valve defects. The tissue-engineered valves would have the advantage of growing with the child, said researchers at the University Hospital of Munich. "If we replace a valve in a child, they will need surgery several times in their lifetime, because they will grow out of the devices, so the ultimate goal is to have a construct which is able to grow with the child and only have to do the surgery once," said study author, cardiac surgeon Dr. Ralf Sodian. Presenting at the American Heart Association's (AHA) annual scientific sessions in New Orleans, Sodian reported that his team took stem cells from umbilical cord blood, stored them for 12 weeks, and then seeded them onto eight heart valve scaffolds. "The whole idea of building a scaffold is a unique idea," said AHA spokesman Dr. Russell V. Luepker, the Mayo professor of epidemiology and community health at the University of Minnesota in Minneapolis. "We generally put progenitor cells in the heart and try to get them to grow muscle cells, and they're sitting in the middle of other cells. But to build a scaffold that

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looks like a heart valve then hope and anticipate that the cord blood cells will take that hint and differentiate, I think is very innovative."16

Bone Healing

Adult stem cells have helped to accelerate the healing of severe leg fractures for five men and four women in Australia. Those involved suffered the worst type of compound bone fractures in serious road accidents, some of whom still could not walk 41 months after their accidents. One man, who suffered a compound fracture and was still using crutches a year later, regained the use of his leg the day after the procedure and is now fully recovered, pain free and regularly runs and plays football. Eight of the ten patients experienced full bone regrowth. The technique was developed by Dr. Richard de Steiger, director of orthopedic surgery at the Royal Melbourne Hospital, who told the media that his team hopes it can be applied to hip replacement procedures as well as accidental injuries.

The technology was developed by the hospital's regenerative medicine company, Mesoblast. Dr. de Steiger said it is between three and five years away from being used in hospitals. In the procedure, bone marrow adult stem cells are harvested from the patient's pelvis in a non-invasive procedure using a needle. The cells are cultivated in a laboratory until they have divided to create 15 billion cells over six weeks. Surgeons then apply the adult stem cells directly to the fractures. One patient in the trial, 36 year-old Anthony Giancola, was walking the following day. "All these patients have avoided the need for having a second operation to get bone from somewhere else in the body. Instead the bone is grown outside the body in a lab," Dr. de Steiger said.17

Liver Cirrhosis

Doctors at Imperial College, London have published results showing improvement in patients with alcoholic liver cirrhosis after treatment with their own adult stem cells. Nine patients had adult stem cells from their blood collected, the numbers were boosted in the lab, and then the cells were injected into their livers. Positive results were seen within one week. Seven of the nine


patients showed significant improvement up to 12 weeks (the extent monitored for this study), with three patients showing almost complete resolution. Dr. Nagy Habib, the senior author, said "We are encouraged that the majority of patients in this study experienced a significant improvement in their liver functions."

These latest results were published in the *American Journal of Gastroenterology*. The new results using adult stem cells to treat liver damage are a follow-up to previous work done by Habib's group and reported in 2006 and 2007. Positive results using adult stem cells to treat liver damage in patients has also been reported by another group in Greece and in Japan.18

**Digestive Tract**

Michael Wenman was the first child in the world to receive a life-saving treatment with bone marrow stem cells. At age five he faced death after an overactive immune system destroyed his digestive tract. Now 12, he was fed intravenously and suffered constant pain until the treatment. His younger brother Matthew, then four, was offered the same procedure when he showed similar symptoms. Both children have since returned to a normal life. They are two of ten children who have now received the treatment.

Two hospitals in the U.K., Great Ormond Street Hospital and Newcastle General Hospital, now offer the adult stem cell therapy. Dr. Neil Shah said the transplant offered a "last chance" for children with this condition, who could only be fed intravenously. He said the stem cells in the marrow seemed to mend the damaged gut and correct the initial imbalance with the immune system. Dr. Shah said the transplant could be seen “as a cure rather than a way of controlling the condition.”19

**Kidney damage**

Adult stem cells are being tested by a Utah research team for prevention and treatment of kidney damage in a Phase I clinical trial. Two patients recently underwent the treatment; a total of 15 patients will be recruited for this trial. This first trial includes patients who have had open-heart surgery, which can sometimes lead to acute kidney damage. The adult stem cells are obtained from

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healthy donor bone marrow and processed so that they are not recognized by the immune system, eliminating the need for tissue transplant matching. The adult stem cells are then injected into the bloodstream of the patient.

Once in the kidney, the cells release signals that protect kidney tissue from damage and stimulate repair. The treatment is based on published work in animals showing that adult stem cells release signals in the kidney that lead to rapid, direct improvement of the tissue. The adult stem cells do not stay in the kidney, but instead transiently provide the signaling that leads to a cascade of protective and repair mechanisms within the organ. This mechanism has been seen in other successful repair of tissues by adult stem cells.20

Pulmonary Hypertension

Lucie Moison has had pulmonary hypertension, a progressive lung disease, for 13 years, and until a year ago was on the waiting list for a lung transplant. Last year, doctors at Jewish General Hospital in Montreal took some of her adult stem cells from her blood, modified them to secrete nitric oxide, and re-injected them into her body. Yvan Depatis also suffered from the disease and was treated with his own modified adult stem cells. Both patients have responded well, with what the doctors termed “absolutely fabulous” results after the experimental treatment. While not yet a cure, the patients have been able to resume many normal activities without suffering from shortness of breath.21

Knee Cartilage Injuries

Scientists have grown a “living bandage” from a patient’s own adult stem cells to heal a common sporting knee injury. Every year about 80,000 men and women in Britain suffer tears to the meniscal cartilage, which acts as a shock absorbing cushion between the bones of the upper and lower leg. Many are in their twenties and thirties. The tears are frequently the result of twisting the leg during jogging, football, rugby, horse riding or skiing. Many sportsmen opt to have the tissue removed. Removal of the loose cartilage allows the athletes to recover, but it leaves bones in the knees exposed and osteoarthritis may develop. Scientists at Bristol University have now managed to heal cartilage tissue in a laboratory with stem cells taken from a patient’s own bone marrow. They used the cells to coat a sponge-like scaffold made from collagen (a fibrous protein),

which was then placed inside the tear in the cartilage. The stem cells pulled the two pieces of torn cartilage together.

The team, led by Anthony Hollander, professor of rheumatology and tissue engineering, will now test the treatment on their first patients. Hollander said: “The stem cells knit across the two sides of the lesion and cause reuniting of the two sides. We hope that in the patient we can reunite the cartilage in a strong enough way to heal the wound completely.” Jonathan Webb, a rugby fullback who played 33 times for England, became a victim of a meniscal cartilage injury in 1989. Webb, 45, who became an orthopedic surgeon specializing in sports injuries after retiring from professional rugby, had cartilage removed but still needs repeated surgery on his knee. He said the stem cell breakthrough “offers the opportunity to rebuild the meniscal cartilage if it cannot be repaired. It may be that the professional sportsmen, who have the most to lose, will drive the technology forward.”

**Windpipe Reconstruction**

Doctors have given a woman a new windpipe with tissue grown from her own adult stem cells, eliminating the need for anti-rejection drugs. The transplant was given to Claudia Castillo, a 30-year-old Colombian mother of two living in Barcelona, who suffered from tuberculosis for years. After a severe collapse of her left lung in March, Castillo needed regular hospital visits to clear her airways and was unable to take care of her children. Doctors initially thought the only solution was to remove the entire left lung. But Dr. Paolo Macchiarini, head of thoracic surgery at Barcelona’s Hospital Clinic, proposed a windpipe transplant instead.

Once doctors had a donor windpipe, scientists at Italy’s University of Padua stripped off all its cells, leaving only a tube of connective tissue. Meanwhile, doctors at the University of Bristol took a sample of Castillo’s bone marrow from her hip. They used the bone marrow stem cells to create millions of cartilage and tissue cells to cover and line the windpipe. Experts at the University of Milan then used a device to put the new cartilage and tissue onto the windpipe. The new windpipe was transplanted into Castillo in June.

Castillo has shown no signs of rejection and is not taking any immune-suppressing drugs, which can cause side effects like high blood pressure, kidney failure and cancer. "I was scared at the beginning," Castillo said in a press

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statement. "I am now enjoying life and am very happy that my illness has been cured." Her doctors say she is now able to take care of her children, and can walk reasonable distances without becoming out of breath. Castillo even reported dancing all night at a club in Barcelona recently. People who might benefit include children born with defective airways, people with scars or tumors in their windpipes, and those with collapsed windpipes.  

**Adult Stem Cells from Wisdom Teeth**

Japanese scientists say they have created human stem cells from tissue taken from the discarded wisdom teeth of a 10-year-old girl. The researchers say their work suggests that wisdom teeth could be a suitable alternative to human embryos as a source for therapeutic stem cells. Research involving stem cells is seen as having the potential to treat many life-threatening diseases. The researchers, based at the National Institute of Advanced Industrial Science and Technology (AIST), say it will be at least five years before their findings result in practical medical applications.

Stem cells have the ability to develop into other kinds of human cells, and experts believe they may eventually lead to treatments for some of the most intractable conditions, such as cancer and diabetes. The AIST researchers said they had identified a form of stem cell in the wisdom teeth which had the capability to develop and be grown successfully into other forms of cell outside the body. The cells they harvested continued to grow in the laboratory for just over a month.

**Flexible Adult Stem Cells from Testes**

Scientists in Germany have shown that adult stem cells with the same flexibility as embryonic stem cells can be grown from human testes tissue. Like embryonic stem cells and iPS (induced pluripotent stem) cells, these human stem cells are pluripotent, showing the ability to grow for long periods in the lab and to form representatives of most or all tissues of the body. This is not the first report of pluripotent stem cells from testes.

A different German team had previously published their results producing such flexible stem cells from mouse testes, and a U.S. group had also published results of producing these flexible stem cells from mice, while a U.S. company had

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claimed they were able to produce flexible stem cells from human testes. However, the German group is the first to publish evidence (online in the journal *Nature*) that such cells can be made from human testes tissue. According to senior author Thomas Skutella, "The advantage these cells have in comparison to embryonic stem cells is that there is no ethical problem with these cells and that they are natural." Skutella is a professor at the **Center for Regenerative Biology and Medicine in Tuebingen**, Germany.  

William L. Saunders is Senior Fellow and Director of the Center for Human Life and Bioethics at Family Research Council. Dr. David Prentice is Senior Fellow for Life Sciences at Family Research Council and a Founding Member of Do No Harm. Jan Ledochowski and Lukas Lucenic were Witherspoon Fellows at FRC in the Summer and Spring of 2008, respectively.

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