Adult Stem Cells: Saving Lives Now

David A. Prentice, Ph.D.
Family Research Council
Washington, D.C., USA
U.S. Senator Arlen Specter:
“Embryonic stem cells are a fountain of youth.”

“Embryonic stem cells have the potential to cure all known maladies.”

Speaker of the House Nancy Pelosi:
“Science has taken us to a place that is Biblical in its power to cure, and that is the embryonic stem cell research.”

“We’re saying science is an answer to our prayers.”
PARTIAL-BIRTH ABORTION?

NO, STEM-CELL RESEARCH
STEM CELL DEPOT

PARTS DEPT.

YA' GOT A FEMUR FOR A '57 CAUCASIAN?
Regenerative Medicine with Stem Cells

Bone marrow stem cells

Damaged heart muscle
Isolation & Culture of Embryonic Stem Cells
(Human-1998; Mouse-1981)

Method patented
U.S. patent held by Univ. Wisconsin

Purported Advantages:
1) Proliferate indefinitely
2) Form any tissue
Promises, Premises, and Published Data…

Claims unsubstantiated for embryonic stem cells

Current or potential embryonic stem cell problems:

• Difficult to establish and maintain
• Difficulty in obtaining pure cultures in the dish
• Potential for tumor formation and tissue destruction
• Questions regarding functional differentiation
• Problem of immune rejection
• Genomic instability
• Few and modest results in animals, no clinical treatments
• Ethically contentious
“Major roadblocks remain before human embryonic stem cells could be transplanted into humans to cure diseases or replace injured body parts, a research pioneer said Thursday night. University of Wisconsin scientist James Thomson said obstacles include learning how to grow the cells into all types of organs and tissue and then making sure cancer and other defects are not introduced during the transplantation. ‘Ultimately, those transplantation therapies should work but it’s likely to take a long time.’.... Thomson cautioned such breakthroughs are likely decades away.”
“Worth noting is that Dr. James Thompson of the University of Wisconsin, who led the team responsible for isolating the first human embryonic stem cell lines in 1998, believes that scientists have overestimated the prospects for transplantation cures using embryonic stem cells. He now focuses his research where he thinks it will be more effective, such as for drug discovery and testing purposes.”
Bergman, Gregory Equities, 55, 10, April 1, 2007

“States will pour more money into this research. We’ll all get more money,” predicts Kevin Eggan, a scientist at Harvard Stem Cell Institute…
“Stem-Cell Advance May Skirt Ethical Debate”, By Gautam Naik, WSJ, June 7, 2007; Page B1
Human embryonic stem cell trial wins approval

Meredith Wadman

In a milestone for a politically charged field, the US Food and Drug Administration (FDA) has approved the world's first clinical trial of a therapy generated by human embryonic stem cells.

Geron, a Menlo Park, California-based company, announced on 23 January that it has won the regulatory agency's approval to launch a small, phase I safety study of a stem cell-derived therapy for spinal cord injury. The publicly traded company has an extensive patent portfolio relating to embryonic stem cell research, and few other competitors; the announcement sent its shares soaring. On Friday, they closed up 36 percent, at $7.09.
Geron Jumps

1/23 12:09 PM ET
In Vitro Fertilization and Frozen Embryos

RAND Survey

Approximately 400,000 frozen embryos in U.S. MOST designated for “family building” Only approx. 11,000 available for research uses Maximum 275 ES cell lines

Hoffman DI et al, Cryopreserved embryos in the United States and their availability for research, Fertility & Sterility 79, 1163-1169, May 2003

“If a more complete match were needed (as is preferred in blood and marrow transplantation), many thousands of hESC lines would need to be available.”

“An open-ended number of hESC lines could theoretically reach into the millions if hESC therapies live up to their potential.”

Civin CI and Rao MS, How many human embryonic stem cell lines are sufficient? A U.S. Perspective, Stem Cells 24, 800-803, 2006
Human Cloning

Good grief! I've been cloned!!
Cloning (Somatic Cell Nuclear Transfer, SCNT)

1. Remove udder cell from white-face sheep
2. Remove DNA from unfertilized egg
3. Fuse cells
4. Early embryo with donor DNA
5. Cloned embryo
6. Implant in surrogate
7. Clone of white-face sheep

"Reproductive cloning" "Therapeutic cloning"
Human somatic cell nuclear transfer (cloning)

The Ethics Committee of the American Society for Reproductive Medicine

American Society for Reproductive Medicine, Birmingham, Alabama

Within 2 years of the announced birth in 1997 of Dolly, the lamb cloned from the mammary cells of an adult ewe, research groups announced that they had cloned mice and calves by using differentiated somatic cells (1–3). In the cloning technique used to produce Dolly, the nucleus of a somatic cell of the ewe
Which comes first – the egg or the cure?

It could happen to you or your loved one:
- Diabetes
- Heart Disease
- Spinal cord injuries
- Parkinson’s disease
- Blindness
- Strokes, AIDS, MS, cancer, among others

Thousands of Americans die everyday from diseases that could potentially be treated - or even cured - using stem cells

Women 21-35 years old needed to donate eggs for stem cell research project.

(All procedures will be carried out at an accredited clinic by certified medical professionals. Travel, hotel and other expense covered)

LET YOUR EGGS BE PART OF THE CURE!
Please donate your eggs. Call 202-315-3736
Egg Harvesting—Health Risk to Women

“Between 0.3 and 5% or up to 10% of women who undergo ovarian stimulation to procure oocytes experience severe ovarian hyperstimulation syndrome, which can cause pain, and occasionally leads to hospitalization, renal failure, potential future infertility, and even death.”
Calla Pappademus, Stanford student who survived problems from egg donation

(see handsoffourovaries.com)
Scientists win right to create human-animal test 'chimeras'

By DAVID DERBYSHIRE
Last updated at 01:18am on 9th October 2007

Scientist will be able to create half-animal half-human embryos for the first time under controversial new rules announced by the Government.

In a dramatic U-turn, Health Minister Dawn Primarolo bowed to pressure from scientists and promised to allow the creation of animal-human "chimeras" for medical research.
Obama Reverses Bush Policy on Stem Cell Research
The Ban on Federal Funding Is Lifted

By Scott Wilson
Washington Post Staff Writer

Obama Lifts Bush’s Strict Limits on Stem Cell Research

The New York Times

Obama Is Leaving Some Stem Cell Issues to Congress

By Sheryl Gay Stolberg
Published: March 9, 2009

Published: March 8, 2009
I DIED WAITING FOR EMBRYONIC STEM CELL RESEARCH TO FIND A CURE. WHAT ABOUT YOU?

I WAS THE EMBRYO
Dolly creator Prof Ian Wilmut shuns cloning

By Roger Highfield, Science Editor

The scientist who created Dolly the sheep, a breakthrough that provoked headlines around the world a decade ago, is to abandon the cloning technique he pioneered to create her.

Prof Ian Wilmut's decision to turn his back on "therapeutic cloning", just days after US researchers announced a breakthrough in the cloning of primates, will send shockwaves through the scientific establishment.

He and his team made headlines around the world in 1997 when they unveiled Dolly, born July of the year before.

But now he has decided not to pursue a licence to clone human embryos, which he was awarded just two years ago, as part of a drive to find new treatments for the devastating degenerative condition, Motor Neuron disease.

Prof Wilmut, who works at Edinburgh University, believes a rival method pioneered in Japan has better potential for making human embryonic cells which can be used to grow a patient's own cells and tissues for a vast range of treatments, from treating strokes to heart attacks and Parkinson's, and will be less controversial than the Dolly method, known as "nuclear transfer."

His announcement could mark the beginning of the end for therapeutic cloning, on which tens of millions of pounds have been spent worldwide over the past decade. "I decided a few weeks ago not to pursue nuclear transfer," Prof Wilmut said.

Most of his motivation is practical but he admits the Japanese approach is also "easier to accept socially."
Human Embryonic Stem Cells -- Without an Embryo
Japanese, U.S. Scientists Reprogram Skin Cells to Act Like Embryonic Stem Cells

By NIDHI THAREJA, M.D.
ABC News Medical Unit
Nov. 20, 2007

Stem cells without embryos: skin cells transformed

By Maggie Fox Wednesday, Nov. 21, 2007; 4:27 AM

WASHINGTON (Reuters) - Two separate teams of researchers announced on Tuesday they had transformed ordinary skin cells into batches of cells that look and act like embryonic stem cells, but without using cloning technology and without making embryos.

Scientists Bypass Need for Embryo to Get Stem Cells

The New York Times

By GINA KOLATA
Published: November 21, 2007

Two teams of scientists reported yesterday that they had turned human skin cells into what appear to be embryonic stem cells without having to make or destroy an embryo — a feat that could quell the ethical debate troubling the field.

Stem Cell Breakthrough Defuses Debate

By MALCOLM RITTER
The Associated Press
Wednesday, November 21, 2007; 2:19 AM

NEW YORK -- Scientists have created the equivalent of embryonic stem cells from ordinary skin cells, a breakthrough that could someday produce new treatments for disease without the explosive moral questions of embryo cloning.

Research teams in the United States and Japan showed that a simple lab technique can rival the complex and highly controversial idea of extracting stem cells from cloned embryos.
• New York Times, Dec. 11, 2007: Inspiration can appear in unexpected places. Dr. Shinya Yamanaka found it while looking through a microscope at a friend’s fertility clinic… At the friend's invitation, he looked down the microscope at one of the human embryos stored at the clinic. The glimpse changed his scientific career.

"When I saw the embryo, I suddenly realized there was such a small difference between it and my daughters," said Dr. Yamanaka, 45, a father of two..."I thought, we can't keep destroying embryos for our research. There must be another way."
induced Pluripotent Stem Cells (iPS cells)  
(Cell Reprogramming)

Add 1-4 genes ± chemicals

Target cell
Oct-4, Sox-2, klf-4, Myc
Oct-4, Sox-2, lin28, nanog

Treated cells behave like pluripotent stem cells
(embryonic-like stem cells)

>150 publications since August 2006
>500 human iPS cell lines reported, all since Nov 2007
Using Embryonic Stem Cells for Patients
“It was not until recently that anyone seriously considered the possibility that stem cells in adult tissues could generate the specialized cell types of another type of tissue from which they normally reside...”

*Stem Cells: Scientific Progress and Future Research Directions*, National Institutes of Health, June 2001; Pg. 26
Current Clinical Trials of Adult Stem Cells

- **Cancers**—Lymphomas, multiple myeloma, leukemias, breast cancer, neuroblastoma, renal cell carcinoma, ovarian cancer
- **Autoimmune diseases**—multiple sclerosis, systemic lupus, rheumatoid arthritis, scleroderma, scleromyxedema, Crohn’s disease
- **Anemias** (incl. sickle cell anemia)
- **Immunodeficiencies**—including human gene therapy
- **Bone/cartilage**—children with osteogenesis imperfecta, knee injuries
- **Corneal scarring**—generation of new corneas to restore sight
- **Stroke**—clinical trials in U.S., U.K., Germany
- **Repairing cardiac tissue after heart attack**—bone marrow or muscle stem cells from patient
- **Parkinson’s**—patient’s own neural stem cells, injected growth factors
- **Growth of new blood vessels**—e.g., preventing gangrene in limbs
- **Gastrointestinal epithelia**—regenerate damaged ulcerous tissue
- **Skin**—grafts grown from hair follicle stem cells, from a few hairs from patient
- **Wound healing**—bone marrow stem cells stimulated healing
- **Spinal cord injury**—clinical trials currently in Portugal, Australia, Ecuador
- **Liver failure**—clinical trials in U.K.
- **Diabetes**—clinical trials in Brazil & U.S.
Improvements in Human Patients

STEM CELL RESEARCH TREATMENTS

ADULT 139

EMBRYONIC 000
Treating Diseases with Adult Stem Cells

IN THEIR LETTER "ADULT STEM CELL TREATMENTS FOR DISEASES?" (28 JULY 2006, P. 439), S. Smith et al. claim that we misrepresent a list of adult stem cell treatments benefiting patients (J). But it is the Letter’s authors who misrepresent the statements and the published literature, dismissing as irrelevant the many scientists and patients who have shown the benefits of adult stem cells.

We have stated that adult stem cell applications have “helped,” “benefited” and “improved” patient conditions. Smith et al.’s Supporting Online Material (2) repeatedly notes patient improvement from these cells (3). We have never stated that these treatments are “generally available,” “cures,” or “fully tested in all required phases of clinical trials and approved by the U.S. Food and Drug Administration (FDA).” Some studies do not require prior FDA approval (4), and even the nine supposedly “fully approved” treatments acknowledged by Smith et al. would not be considered “cures” or “generally available” (5) to the public at this stage of research.

The insistence that no benefit is real until after FDA approval is misplaced. Such approval is not a medical standard to evaluate patient benefit, but an agency determination that benefits outweigh risks in a broad class of patients. Physicians and patients use an evidentiary standard. Our list of 72 applications, compiled from peer-reviewed articles, documents observable and measurable benefit to patients, a necessary step toward formal FDA approval and what is expected of new, cutting-edge medical applications.

Smith et al. also mislead regarding citations for testicular cancer and non-Hodgkin’s lymphoma, referring to “[the reference Prentice cites...” as though only one reference existed in each case, and not mentioning four other references that, according to their own SOM, show “improved long-term survival” of patients receiving adult stem cells. There are currently 1238 FDA-approved clinical trials related to adult stem cells, including at least 5 trials regarding testicular cancer and over 24 trials with non-Hodgkin’s lymphoma (5). They also disregard studies showing successful stimulation of endogenous cells for Parkinson’s.

The ethical and political controversy surrounding embryonic stem cell research makes scientific claims especially prone to exaggeration or distortion. All such claims should receive careful scrutiny...

—Prentice and Tarne

References and Notes
1. Posted at the web site of Do No Harm: The Coalition of Americans for Research Ethics: www.stemcellresearch.org
2. Available at www.sciencemag.org/cgi/content/full/312/5774/220.
3. See Table 5 in the Supporting Online Material on Science Online at www.sciencemag.org/cgi/content/full/313/5532/886/DC1; seven applications were not analyzed in the editorial update of 12 July 2006.
4. E.g., for studies done outside the United States, FDA approval is irrelevant; also see, e.g., 21 CFR 217.10, 21 CFR 217.35, 21 CFR 217.13.
5. See www.clinicaltrials.gov/ctn/search?term=stem-cell (accessed 10 October 2006); initial search shows clinical trials recruiting patients; click box in upper left to show all trials, including those no longer recruiting patients.
MEDICAL TREATMENTS FOUND BY USING:

ADULT STEM CELLS: 73
EMBRYO STEM CELLS: 0

STOP LETHAL EMBRYO RESEARCH IN UCC
<table>
<thead>
<tr>
<th>Rank</th>
<th>Status</th>
<th>Study</th>
</tr>
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<tbody>
<tr>
<td>1</td>
<td>Completed</td>
<td>Haploidentical Stem Cell Transplant for Treatment Refractory Hematological Malignancies</td>
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<td></td>
<td></td>
<td>Conditions: Acute Lymphoblastic Leukemia (ALL); Acute Myeloid Leukemia (AML); Secondary AML; Myelodysplastic Syndrome (MDS); Secondary MDS; Chronic Myeloid Leukemia; Juvenile Myelomonocytic Leukemia (JMML); Paroxysmal Nocturnal Hemoglobinuria (PNH); Lymphoma; Non-Hodgkin; Hodgkin Disease</td>
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<tr>
<td></td>
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<td>Interventions: Procedure: Stem Cell Transplantation; Device: Miltenyi Biotec ClinMACS; Drug: Systemic chemotherapy and antibodies</td>
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<td>2</td>
<td>Recruiting</td>
<td>Stem Cell Transplant in Sickle Cell Disease and Thalassemia</td>
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<tr>
<td></td>
<td></td>
<td>Conditions: Sickle Cell Disease; Beta Thalassemia</td>
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<tr>
<td></td>
<td></td>
<td>Interventions: Drug: Busulfan; Drug: Fludarabine; Drug: Alumetzumab; Procedure: Allogeneic stem cell transplant</td>
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<tr>
<td>3</td>
<td>Completed</td>
<td>Partially Matched Stem Cell Transplantation for Patients With Refractory Severe Aplastic Anemia or Refractory Cytopenias</td>
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<tr>
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<td>Conditions: Anemia, Aplastic; Amegakaryocytic Thrombocytopenia; Diamond-Blackfan Anemia; Kostmann Syndrome</td>
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<tr>
<td></td>
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<td>Intervention: Procedure: Allogeneic stem cell transplant</td>
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<tr>
<td>4</td>
<td>Active, not recruiting</td>
<td>Trial of Allogeneic Stem Cell Transplants From HLA Compatible, Related and Unrelated Donors After a Myeloablative Preparative Regimen With Hyperfractionated TBI, Thiota and Fludarabine For Adult Patients With Lymphohematopoietic Disorders</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Conditions: Allogeneic Stem Cell Transplant; Leukemia; Non-Hodgkin; Lymphoblastic Lymphoma; Myelodysplastic Syndrome; Paroxysmal Nocturnal Hemoglobinuria (PNH)</td>
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<tr>
<td></td>
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<td>Intervention: Drug: cytoreductive regimen followed by a CD34+- selected allogeneic stem cell transplant</td>
</tr>
<tr>
<td>5</td>
<td>Active, not recruiting</td>
<td>Sm-EDITMP and Autologous Peripheral Blood Stem Cell Transplant for Breast Cancer Patients With Bone Only Metastases</td>
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<tr>
<td></td>
<td></td>
<td>Conditions: Breast Cancer; Bone Metastases</td>
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<tr>
<td></td>
<td></td>
<td>Interventions: Drug: 153 Sm-EDITMP; Behavioral: Questionnaire; Procedure: Stem Cell Transplant</td>
</tr>
<tr>
<td>6</td>
<td>Recruiting</td>
<td>Stem Cell Transplant for Immunologic or Histiocytic Disorders</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Conditions: Hemophagocytic Lymphohistiocytosis; X-Linked Lymphoproliferative Disorders; Chediak-Higashi Syndrome; Griscelli Syndrome; Immunologic Deficiency Syndromes; Langherhans-Cell Histiocytosis</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Interventions: Procedure: Stem Cell Transplant; Drug: Fludarabine, melphalan, ATG or Campath</td>
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What if you could do this after a heart attack?

ATLANTA – Emory University physicians are revolutionizing heart care by using a patient’s own stem cells to restore damaged heart muscle after a heart attack. It’s one of hundreds of treatments developed at Emory, where doctors don’t just practice medicine; they advance it. For more information, visit www.emoryheart.org.
Carol Franz before & after treatment with her own bone marrow adult stem cells for multiple myeloma.
Kaitlyn McNamara had a new functional bladder constructed from her own adult stem cells.
Helen Thomas, 80, was treated for peripheral artery disease with her own adult stem cells. The transplant saved her leg from amputation.
Jacki Rabon, 18, is walking with braces on a parallel bar six months after undergoing adult stem cell surgery to repair a spinal cord injury that left her paralyzed. *Photo by Becki Rabon*

Dennis Turner.
Treated for Parkinson’s with his own brain adult stem cells.
Doug Rice has seen dramatic improvements in his heart after treatment with his own bone marrow adult stem cells.

David Foege, treated for heart failure with his own blood adult stem cells, has gone from an ejection fraction of 15% to 58%
Adult Stammzellen
Therapiemöglichkeiten bei Herz- und Kreislauffrankungen

Adult stem cells
Potential therapies in cardiac and vascular diseases
Roland Henrich suffered a stroke and was treated within 24 hours with his own bone marrow adult stem cells. Within 11 days after treatment he showed no signs of paralysis and said his first word since the stroke.
Jaider Abbud has gone over a year without insulin or other medication after treatment with his own bone marrow adult stem cells for juvenile diabetes.

Dr. Julio Voltarelli (left) and Dr. Richard Burt (right), with one of their patients successfully treated for Type I Diabetes using the patient’s own bone marrow adult stem cells.
Amy Daniels (systemic sclerosis), Barry Goudy (multiple sclerosis), and Jill Rosen (lupus) prepare to tell a Congressional briefing about their successful treatments using adult stem cells.
Claudia Castillo had a complete new trachea grown using her own adult stem cells. The transplant saved her lung and she is now doing well.
Stephen Sprague, treated in 1997 for chronic myelogenous leukemia, with umbilical cord blood stem cells.

Erik Haines, now 14 years old, was diagnosed with Krabbe’s Disease in 1994 and was the first patient to receive a cord blood transplant for this rare, inherited metabolic disease.
Diana Tirpak was treated with donor cord blood for her leukemia.
Nathan Mumford was able to find an adult stem cell match to treat his leukemia with cord blood.
Good News on Multiple Sclerosis with Adult Stem Cells

by David Prentice
May 11, 2010

A groundbreaking new study published in the last week provides more good news for treatment of multiple sclerosis (MS) with adult stem cells. Researchers at the University of Bristol used patients’ own adult stem cells to treat their MS.

In a Phase I clinical trial, six patients with MS were treated with their own bone marrow adult stem cells and their progress followed for one year. The treatment appeared to stabilized the patients’ condition and showed some benefits. As one measure of the success of the procedure, damaged nerve pathways were able to carry electrical pulses more effectively after the treatment.

Multiple sclerosis is an incurable disease, with the patients own immune system attacking the central nervous system and eventually leaving many patients in a wheelchair.

One of the most encouraging aspects of this trial was the elegantly simple procedure. Patients reported to the hospital and had bone marrow adult stem cells removed, the cells were filtered, and then given back to the patients intravenously. The patients went home before the end of the day.

The research team is led by Professor Neil Scolding, at the University of Bristol and North Bristol NHS Trust. Professor Scolding said:
Getting Hip, Boning Up with Adult Stem Cells

by David Prentice
May 12, 2010

Dr. Thomas Einhorn at Boston Medical Center has now treated about 50 patients for painful degenerative hip disease with the patient’s own adult stem cells. One of those patients, Jose Belsol, was training for a triathlon when diagnosed. Einhorn used Jose's bone marrow adult stem cells, injected into the hip, to help generate new bone. Jose now has hope that he will be able to compete in sports again. Dr. Einhorn notes:

“As long as I can win the race against time to replace that tissue with cells that can make bone and prevent that collapse from happening, Mr. Belsol should be OK.”

UK doctors have also had success using adult stem cells to repair hip bones.

A Korean team led by Dr Seok-Jung Kim recently published their data using cells cultured from bone marrow to speed bone healing after fractures. The 31 patients who received injection of their own cells showed significantly faster bone healing compared with the 33 patients who healed without use of added cells.

Dr. Lew Schon has also used bone marrow adult stem cells to speed bone healing. For one of his patients, Rob Attori, the treatment decreased healing time from the usual 3-6 months in a brace down to nine weeks, in time for him to make a 4,000-mile bike ride he had planned. Rob now works with Dr. Schon on stem cell research.
Boy Gets New Windpipe Made With His Adult Stem Cells

by David Prentice

May 14, 2010

A ten-year-old boy is now breathing easy, thanks to a world first transplant using a new windpipe grown using his adult stem cells. The young boy was born with a rare condition called Long Segment Tracheal Stenosis, with a narrow windpipe that does not grow and restricts breathing. He had undergone previous surgeries to widen his windpipe but the condition had become life threatening. A team of British and Italian doctors developed a new technique to treat the young boy’s life-threatening condition. They took a donor trachea, stripped it down to the cartilage scaffolding, and then injected adult stem cells from the boy’s bone marrow. The stem cell-coated organ was then implanted in the boy. Over time the adult stem cells will cover the windpipe; using his own stem cells means there is no transplant rejection problem.

The major step forward in this case, is that instead of re-growing the organ with adult stem cells in the laboratory for months until it is fully formed, the cells were put into the trachea just before implanting it. The team of British and Italian scientists described the procedure as a breakthrough for its simplicity in using the ‘ideal laboratory’ of the human body to rebuild the organ.
Building Bone for Wounded Warriors
by David Prentice
June 17, 2010

Dr. Kent Leach at UC-Davis has been working on strategies to regenerate functional tissues, to repair or replace damaged tissues. Leach's lab has developed methods combining adult stem cells and composite gel matrices to stimulate tissue regeneration, including growth of bone.

Dr. Leach has now received a Hypothesis Development Award from the U.S. Army to explore an approach to speed bone healing. The research will help development of effective treatments for wounded soldiers or veterans struggling with slow-healing or non-healing bone damage. Soldiers in combat suffer massive wounds to bone and tissue, with about 70% of war wounds as musculoskeletal injuries, over half of those being to the arms and legs.

Dr. Leach's approach will use adult stem cells derived from human adipose (fat) tissue, embedding the cells into a special hydrogel and implanting directly in the injured site. The approach offers the advantages of using the patient's own easily-harvested cells, and increasing the concentration of cells and bone-building substances at the site of the injury.
Gene-Engineered Adult Stem Cells to Fight HIV

by David Prentice
June 17, 2010

Scientists at City of Hope in Duarte, California have shown that it may be possible to genetically engineer a patient’s own adult stem cells to fight HIV. Four patients with AIDS received their own adult stem cells to treat lymphoma, including some of their cells that had been engineered with three genes to fight off HIV infection. One of the gene therapy targets was a protein receptor on immune cells (CCR5) that HIV binds to when infecting a cell, the idea being to prevent infection of the engineered stem cells. The other two engineered genes were designed to attack viral RNA that might make it into the cell, preventing production of viral protein.

While the dose of engineered stem cells was too low to produce an effect on the patients’ viral load, the study showed no adverse effects from the procedure and that the cells survived, engrafted, and continued producing the engineered genes for up to two years after the transplant for three of the four patients.

A previous study had treated leukemia in an AIDS patient using donor adult stem cell transplant, in which the donor was selected specifically for lack of the CCR5 receptor on the donor stem cells. The patient recovered from the leukemia and also exhibited no sign of HIV.

The current study was published in Science Translational Medicine.
Sight Restored to Blinded Patients using Their Own Adult Stem Cells

by David Prentice
June 18, 2010

Italian scientists report that they have restored sight to patients blinded by chemical burns using the patient’s own adult stem cells. The team treated 112 patients blinded in one or both eyes; some of whom had been blind for years. Adult stem cells were taken from the edge of a patient’s eye and cultured on fibrin, then the cell layers transplanted onto the damaged eyes. The adult stem cells produced healthy corneas and functioning eyes. Some patients regained sight within two months, while for others with deeper injuries the process took a year before vision was restored. Patients were followed up to ten years after the transplant. After a single transplant, 69% of patients regained vision; in some cases a second transplant occurred, with a total success in 77% of patients and partial vision restoration in 13% of patients. The long-term restoration was an especially encouraging success of the study.

Lead researcher Dr. Graziella Pellegrini, of the University of Modena, said:

“The patients, they are happy, even the partial successes. We have a couple of patients who were blind in both eyes. Can you imagine for these patients the change in their quality of life?”
Adult STEM CELLS Saved My Life

Education and awareness campaign