Umbilical Cord Blood & Cord Blood Stem Cell Therapy: Is Something Amiss?

By
Choctaw Doc

One of the most frequent complaints I hear when chatting with folks about the promise and limitations of umbilical cord stem cells in addressing various non-blood borne human maladies is the seeming intransigence of “the feds” (NIH and FDA) when it comes to allowing doctors to employ these cells in hospitals and medical offices here in the United States. Many resent the fact they must travel to Mexico, Thailand, Holland and other countries to get what they perceive is a fundamentally safe though admittedly experimental treatment for conditions such as chronic stroke, cerebral palsy, ALS, multiple sclerosis, macular degeneration and a host of other human afflictions.

The FDA, as we all know, has a mandate to make sure that the pharmaceuticals, surgical techniques, medical devices and biologic agents and vaccines that reach the medical consumer are safe and effective. In this regard they have had a great deal of success and their fair share of failures such as the fenfluramine-dexfenfluramine (Fen-Phen) Celebrex® and Vioxx® drug scandals. And their ranks are certainly not immune to corporate influence and even corruption, as we all learned when it was revealed that a great many researchers at the NIH were being paid handsome consultancy fees from major pharmaceutical companies.

Reform is obviously needed and is in fact underway. Senior NIH officials are no longer allowed to take consultancy fees from private firms and limitations were imposed on regular staff as well. But is this enough? Many of the people I speak with feel that something is amiss at the NIH and FDA, and they point to cord blood itself as a prime example. Yes, these are typically individuals who have a stake in being able to have cord blood or cord blood stem cell treatments done on themselves or a relative or friend. But even so, these folks are not blind or stupid; they have more-often-than-not done their homework in terms of scouring and analyzing the scientific literature and a great many report seeing a disturbing pattern: In-a-word, they see the guardian of public health -- the FDA -- seemingly intent on standing in the way of progress. They also detect a kind of tunnel vision within the scientific community as well. Is this perception merely a species of “conspiratorial thinking” or post 9-11 paranoia? Or is there indeed

something amiss going on? And if there is something amiss, what should be done about it?

The use of cord blood in the American medicine goes back at least 20 years. Scientists found that the stem cells in cord blood could be used to create a whole new bone marrow system in people whose own marrow was defective in some way. For example, in many folks with leukemia it was found that eradicating the patient's bone marrow with chemotherapy or radiation, then infusing cord blood often led to the genesis of healthy, cancer free marrow. Other blood borne and some immune system applications followed. Today no credible expert disputes the therapeutic power cord blood stem cells hold when it comes of addressing diseases and disorders that arise because of some defect in bone marrow function. Recently, advanced multiple sclerosis patients have the option of having their bone marrow replaced in a fashion not unlike that of leukemia victims. As this is highly experimental, patients must cough up $100,000 or more the many centers that do this procedure typically charge. As MS is, at least in part, an autoimmune disease in which immune cells generated in bone marrow attack the myelin sheath that insulate nerves in the patient's central nervous system, it makes sense that this approach might work. And there are patients on record so treated whose progressive MS is now in remission.

This body of clinical application and the research underlying it leaves little doubt but that cord blood and cord blood stem cells have imminent utility in ameliorating and in some instances curing some bone marrow-spawned or related diseases and disorders plus various immune system-related disorders. But what of non-blood borne diseases and disorders?

According to many mainstream researchers and stem cell experts, cord blood and cord blood stem cells will have little if any salutary effect in neurologic, eye, circulatory and other conditions. Since embryonic stem cells do have demonstrated plasticity, which is to say the ability to form the multitude of tissues and such that make up the human body, this has become the focus of much stem cell related research and state, federal and private funding here in the States. But in the rush to lay hold of the promise inherent in embryonic stem cells have many mainstream scientists and the NIH and FDA prematurely written off the potential for cord blood and cord blood stem cells to significantly improve or in some instances turnaround many non-blood borne conditions? Have one or more of these players intentionally or unintentionally placed bias above scientific curiosity and openness? Many scientists and a great many laypeople think so.

Excerpted section from a government sponsored paper -- What one scientist says about umbilical cord stem cells:

David A. Prentice, Ph.D.
Professor of Life Sciences at Indiana State University,
Terre Haute, Indiana

Use of umbilical cord stem cells has seen increasing interest, as the cells have been recognized as a useful source for hematopoietic transplants similar to bone marrow stem cell transplants, including for treatment of sickle cell anemia.[164] Cord blood shows decreased graft-versus-host reaction compared to bone marrow,[165] perhaps due to high interleukin-10 levels produced by the cells.[166] Another possibility for the decreased rejection seen with cord blood stem cell transplants is decreased expression of the beta-2-microglobulin on human cord blood stem cells.[167] Cord blood can be cryopreserved for over 15 years and retain significant functional potency.[168] Cord blood stem cells also show similarities with bone marrow stem cells in terms of their potential to differentiate into other tissue types. Human cord blood stem cells have shown expression of neural markers *in vitro*,[169] and intravenous administration of cord blood to animal models of stroke has produced functional recovery in the animals.89,[170] Infusion of human cord blood stem cells has also produced therapeutic benefit in rats with spinal cord injury,[171] and in a mouse model of ALS.[172] A recent report noted establishment of a neural stem/progenitor cell line derived from human cord blood that has been maintained in culture over two years without loss of differentiation ability.[173] Several reports also note the production of functional liver cells from human cord blood stem cells.[174] Additional differentiative properties of human umbilical cord blood stem cells are likely to be discovered as more investigation proceeds on this source of stem cells.

From **Appendix K** http://bioethicsprint.bioethics.gov/reports/stemcell/appendix_k.html, Adult Stem Cells*, Monitoring Stem Cell Research* Table of Contents The President's Council on Bioethics, Washington, D.C.
January 2004 http://www.bioethics.gov/

From Stem Cell Basics – The official *National Institutes of Health* resource for stem cell research

C. What is known about adult stem cell differentiation?

Adult stem cell plasticity and transdifferentiation. A number of experiments have suggested that certain adult stem cell types are **pluripotent**. This ability to differentiate into multiple cell types is called plasticity or transdifferentiation. The following list offers examples of adult stem cell plasticity that have been reported during the past few years.

- Hematopoietic stem cells may differentiate into: three major types of brain cells (neurons, oligodendrocytes, and astrocytes); skeletal muscle cells; cardiac muscle cells; and liver cells.

- Bone marrow stromal cells may differentiate into: cardiac muscle cells and skeletal muscle cells.

- Brain stem cells may differentiate into: blood cells and skeletal muscle cells.

Current research is aimed at determining the mechanisms that underlie adult stem cell plasticity. If such mechanisms can be identified and controlled, existing stem cells from a healthy tissue might be induced to repopulate and repair a diseased tissue

http://stemcells.nih.gov/info/basics/basics4.asp

This sounds all fine and good. On the one hand we have Dr. Prentice's statement that cord blood and cord blood stem cells has improved stroke, spinal cord injury and ALS in animal models. On the other, we see that adult stem cells including hematopoietic (bone marrow and cord blood) progenitor/stem cells can be transformed into non-blood and immune related cells such as neurons, cardiac muscle and liver cells CORD BLOOD STEM CELL PLASTICITY, although admittedly we need to understand more about the mechanisms that bring this about so that we can control it and target repair in the human body.

But what of the merit, if any, of cord blood or cord blood stem cells that are simply infused, injected or implanted in people? That is, put in without being primed or differentiated outside (*ex vivo*) the recipient's body? Shouldn't we expect salutary effects in at least some non-blood borne conditions given what has been seen in rats and other lab animals? Doesn't it make sense that if these cells can be given to an animal – not provoke rejection or any appreciable adverse reaction – and bring about repairs in brains ravaged by induced strokes or spinal cord injury or ALS -- that this scenario might hold true in humans also?

Well, this is exactly what researchers at *Steenblock Research Institute* have been documenting since early 2003.* While their evidence to-date is tentative and far from qualifying as rigorous in the hard science sense, they feel there are sufficient specific repetitive post-stem cell treatment improvements in various medical conditions to warrant pursuing formal randomized clinical trials. For example:

Three (3) children with cortical blindness due to optic nerve atrophy or hypoplasia experienced partial resolution of this within 90 days following a single infusion of 1.5 million CD34+/CD133 umbilical cord stem cells (in Mexico). These children varied in age, sex and degree of neurologic impairment – and yet all 3 began tracking objects within 90 days of their respective hUCSC treatments.

One of these children, Adam Susser, was not only cortically blind prior to hUCSC therapy, but could not get about well or speak either. Four stem cell treatments later he is doing all three. Adam's father, consumer affairs attorney Gary Susser, has set up the ADAM SUSSER FOUNDATION devoted to helping children like his son.

A 5 minute Sinclair Broadcast Group video on Adam can be readily access online by going to STEM CELL THERAPIES.ORG Click the "Resources" link – drop down to #12 – and click the "Watch Stem Cell Video Link". It is also on YouTube at http://bit.ly/zDWSss.

Also:

At least 5 children with intractable seizures are reported to have experienced a significant reduction in frequency and intensity following hUCSCT (Injected and IV). Many became seizure free within months following their treatment.

Many adults with visual impairment due to stroke or macular degeneration have experienced partial recovery of their ability to see following catheter infusions of various kinds of cord blood derived stem cells, e.g., CD34+AC133, mesenchymals, CD34-AC45+, primitive neurogenic progenitors, etc.

During 2004 SRI took part in a pilot study conducted by Fernando Ramirez, MD in Mexico in which 8 children with cerebral palsy received a single injection each of 1.5 million CD34+/CD133 cord blood stem cells. None of the children experienced any symptoms of rejection or graft v. host (Consistent with what has been noted by various researchers throughout the world). All eight showed some improvement in mobility and/or cognitive function. Six children (75%) were rated as improving in muscle tone,

hip movement, leg movement, rolling to the side, balancing while sitting and balancing while standing by the end of the six month follow up.

A paper based on this study was accepted by the free access journal, *Medical Hypotheses & Research* and can be accessed by clicking this link: RAMIREZ PILOT STUDY - CORD BLOOD STEM CELLS AND CEREBRAL PALSY

In addition, some of the children who participated in this study have had their stories showcased in "Umbilical Cord Stem Cell Therapy" (Basic Health Publications, 2006). This book is probably the first book on cord blood stem cell therapy ever written for laypeople.

Similar case histories are being reported by clinics and hospitals that do cord blood stem cell therapy in Thailand, China, Germany, England and elsewhere.

Even cord blood itself has shown efficacy in ameliorating some non-blood born disorders. One example is a body of separate lab experiments carried out by Dr. Normal Ende (UMDNJ-New Jersey Medical School) and Dr. Robert Brown at Harvard Medical School in which cord blood proved of benefit in treating ALS in animal models. Following on the heels of this, Mitchell Ghen, D.O. in Atlanta and Daniel Cosgrove, M.D., in La Quinta, CA. found that administering multiple units of seemingly unmatched cord blood to patients with ALS (Lou Gehrig's disease) resulted in improvements in many of the patient's ability to get about, swallow and such (There were also some who reported no improvement and possibly disease progression). Both men had approval from an IRB (Independent Review Board) – committees that pass muster on the ethics part of research studies – but no FDA issued permit to do a clinical study (Investigational New Drug permit - IND). As a result, during March 2003 federal agents got a warrant to obtain Dr. Ghen's medical records, after which he (Ghen) called a halt to this particular line of work. He had treated at least forty-three (43) patients at the time his cord blood treatment program ceased operating.

And at the University of Kansas, Dr. Kathy Mitchell along with Harvard researcher Dr. Denise Faustman found evidence in their NIH funded studies that cord blood stem cells brought about significant improvements in lab models of stroke, kidney disease and diabetes.

The Response of the NIH, Federal Regulatory Agencies & Mainstream Researchers

In the world of biomedicine, the question of whether a particular drug or procedure is effective or not is settled in the arena of science – namely, well designed and executed clinical studies. One doesn't dismiss a treatment or procedure that has a rational foundation (i.e., doesn't violate established scientific laws or principles) *a priori* – which is to say, before conducting tests. But this has been exactly what many experts in stem cell biology have done when it comes to the observations and data collected and reported by SRI. Rather than suggest the need for controlled studies to determine whether or not the clinically significant improvements reported in scores of patients treated with purified cord blood stem cells in Mexico for non-blood maladies pan out, many of these scientists have dismissed the entire treatment as "a scam".

This is not the kind of degree of informed open-mindedness one would expect of highly placed researchers in major institutes and universities.

The level of cautious open-mindedness among officials in many federal regulatory agencies appears similarly lacking or compromised. Drs. Ghen and Cosgrove's use of cord blood infusions to treat ALS (cited above) provides one such example:

As was mentioned previously, there were improvements in some of the ALS patients treated, while others saw little or nothing or seemed to progress. There were also side effects reported: Three of Dr. Ghen's patients reported passage of dark urine starting a few hours after infusion and continuing for 24 hours or so. Persistent dark urine can signal hemolytic anemia and/or kidney damage. Additionally, one patient had heart palpitations that occurred for 30 seconds about three to four times a day and lasted one week after the treatment.

But perhaps because there were salutary results and no discernable permanent harm done, the FDA expressed a willingness to allow individual patients to be treated with cord blood on an individual basis. Treating physicians would apply for an Emergency IND – something often granted to terminally ill patients who want to receive an experimental drug or procedure.

So far, so good.

However, when Dr. Nizar Souayah, personal physician to ALS sufferer and decorated war veteran, Major Michael Donnelly (ret) – a man who'd received a cord blood treatment and had a good response -- applied for an IND, it was turned down. The intervention of four congressmen and a US Senator on behalf of Major Donnelly was ultimately rebuffed by FDA officials. This rather quixotic and perplexing state-of-

affairs has been ably documented by James Kelly, a spinal cord injured medical advocate and journalist on his website JAMES KELLY WEBSITE. What follows is part of what Mr. Kelly posted on his website concerning the Donnelly case:

“ALS patient Major Michael Donnelly (ret.), a decorated fighter pilot from Desert Storm, received cord blood in 2002 through the kind assistance of the White House Office for Veteran's Affairs. His first treatment, which led to partial reversal of paralysis, was attended and approved by the V.A.'s Surgeon General. Today, 18 months since the FDA stopped his treatments, Major Donnelly cannot speak, breathe, eat, or move at all except to blink.”

Does protecting public health include denying dying patients access to a treatment that proved of benefit during their first go-round?

Some patient advocates I have spoken with concerning the actions of the NIH and FDA think there is something sinister afoot, indicative of corruption or even conspiracy to keep cord blood stem cell research on the proverbial back burner. In this writer’s opinion, the events and actions that have played out against cord blood and cord blood stem cells do not reflect a conspiracy but, rather, a way of doing business; a mechanism that works but which can lead to blind spots, tunnel vision and bottlenecks. Case in point: Dr. Kathy Mitchell, a researcher at the University of Kansas-Laurence and Harvard scientist Dr. Denise Faustman. Both of these accomplished scientists have conducted research that points to the potential of cord blood stem cells for treating kidney disease, stroke and diabetes. Although the NIH did fund their basic research – Petri dish work – when it came time to get additional grants to cover animal studies, doors that were once wide open were closed. The scientists who were charged with reviewing Mitchell and Faustman’s grant applications ignored the wealth of data that these two scientists furnished and instead awarded money to embryonic stem cell research projects. Interestingly, these peer reviewers were also competing against Dr. Faustman for different grants.

"The review is totally different than every other segment of the economy," Faustman says. "If every time you wanted to open a dry cleaners you had to go to 90 percent of your competitors and get a consensus, what would be the chance you'd be able to open a profitable business?"

"I think people who want embryonic stem cells just don't want [alternatives] to work," Faustman said.

[Quotes from “Miracle Cells,” February 8, 2005, *World Magazine* MIRACLE CELLS]

Have things changed since Mitchell and Faustman ran head-long into the NIH’s funding turn down? You tell me: A NIH clinical trials database search done during May 2006 produced 98 trials, only one of which appears to be for a *non-blood born* disorder (Diabetes). A subsequent search done on April 13th 2009 – again using “cord blood” as the key word – produced 211 trials, only four of which appear to be for *non-blood born* disorders (And 2 of these had to do with the effects of cord blood serum on corneal healing): NIH CLINICAL TRIALS - CORD BLOOD STEM CELLS

Ecce Signum

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*I was an employee (staff theoretician and senior science writer) at SRI from March 2003 until June 2007. I am currently working as a writer for several firms. Readers can be reach me by e-mail at biotheoretician@gmail.com.

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