



# Complex Child E-Magazine

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## Umbilical Cord Blood Treatments for Children with Cerebral Palsy by Susan Agrawal

Over the past year or so, the popular media has featured several little boys and girls with cerebral palsy who have made unbelievable progress after receiving umbilical cord blood infusions of their own saved cord blood. Some of these children have completely overcome all signs of motor impairments, while others have started speaking, eating, and moving in more typical ways. Naturally, parents of children with cerebral palsy want more information about these treatments. This article will provide a brief introduction to these treatments, who might be eligible for them, and how they may be transformed in the future to be even better and more widely applicable.

### **Current Treatments and Results in the United States**

At this time, a study is underway at Duke University led by Dr. Joanne Kurtzberg, director of the Pediatric Blood and Marrow Transplant Program, to infuse a child's own umbilical cord blood in hopes of treating children with cerebral palsy. About fifty children have had the treatment thus far, with an eventual goal of tracking them over two or more years. As of this writing, no research has been published on this topic, preliminary or otherwise.

Currently, only children who have had their umbilical cord blood banked are eligible for the study. Families who have donated their baby's cord blood to a public bank may be eligible if it is possible to locate the baby's donated blood. It is not possible to use the cord blood of a sibling or other related or unrelated donor at this time. Since cerebral palsy is considered a static and non-life-threatening disease in and of itself, the risks of using a related or unrelated donor are simply too high. Using cord blood from a donor requires the child to undergo chemotherapy to destroy the immune system, a procedure that is very risky. In addition, children require anti-rejection medication and may experience serious graft-versus-host disease or rejection complications. Because these risks are only considered acceptable in life-threatening diseases, related or unrelated cord blood is saved for diseases like leukemia, Krabbe disease, and similar conditions.

The treatment itself is quite simple.<sup>1</sup> The cord blood, rich with stem cells, is retrieved from the bank and DNA analysis is performed to verify that it is indeed the umbilical cord blood of the child. The cells are then washed and prepared for infusion. Next, the

child is infused with the cells through a standard IV over a short period of time, usually less than an hour. After some IV fluids and monitoring, the procedure is complete.

The risks of the procedure are extremely low since the infusion is made up of the child's own cells. Some children may react to preservatives from the cells, but otherwise few side effects are expected.

After infusion, the stem cells travel through the bloodstream and are able to pass through the blood-brain barrier into the brain.<sup>2</sup> It is not known exactly how the cells know where to go, but stem cells typically "find" areas of disease and have several effects on the brain. These include cell-based repair and neuroprotection, such as support of neurons, protection of neurons, promotion of growth of neurons, and remyelination (regrowth of sheaths around nerves) if necessary. In addition, the stem cells begin to multiply and spread throughout the body to needed areas.

After the infusion, it may take some time for the cells to engraft and begin to have effects. Most children begin to see some effects within a week, and further changes in the first three months. These may include increased strength and mobility, improved feeding, reduction of seizures, improved speech, and improved vision, as well as progress in other areas.

For most children, this is not a miracle cure. While all children have seen improvements, the majority of children only showed modest effects.<sup>3</sup> The principal researcher has noted that in some cases it is impossible to know whether the treatment caused the improvements, or whether they were caused by some other effect, such as higher expectations, placebo effect, or concurrent therapies.

Some children have shown dramatic effects, recovering or gaining skills and showing incredible progress. One child shows few lingering effects of cerebral palsy, while several others are now walking and talking. It appears that children who are treated earlier in life, preferably shortly after birth, or who have less serious cerebral palsy may benefit the most from these treatments.

### **Should You Bank Your Child's Umbilical Cord Blood?**

With such a promising treatment becoming available, many parents want to know whether or not they should collect their child's cord blood. The simple answer is yes, all parents should have their newborn's cord blood collected after birth.

But there is no simple answer to what type of bank you should use. Currently, there are two types of banks: public and private. Public banks collect and store cord blood free of charge, while private banks are quite costly, often about \$1200-2200 the first year, and \$100-200 each year thereafter.

There have been concerns about private banks beyond the cost, which is undeniably prohibitive for most families. The majority of physicians and physician groups, including the American Academy of Pediatrics, oppose private banks, accusing them of providing misleading information, false hope, and using a new mother's emotional vulnerability to profit financially. Many of these private banks are for-profit enterprises, meaning they make money off of what may be life-saving stem cells. Very few units from these banks have ever been used, since the number of uses of cord blood in and of itself is small, and one's own donation typically cannot be used for most conditions, including childhood leukemia and other malignancies that often preexist in the umbilical cord cells. There are also questions regarding the usefulness of a single umbilical cord cell donation in a larger child or adult, which may mean that the cells end up unusable for older or larger individuals. While the amount of umbilical cord blood is likely to be more than adequate for a baby or young child, it is not typically a great enough quantity for individuals who weigh over about 130 pounds.<sup>4</sup> It is estimated that only 12% of current samples would be suitable for a large child or adult, though research is underway to expand the number of cells and increase the quantity. With such limited uses and such a high cost, private banking is probably unrealistic and unnecessary for most families.

Direct donation, or banking a sibling's cord blood for the express purpose of treating another sibling, is usually available at a lower cost or no cost at all for families with a child who may be a candidate for transplant. These programs are available through both public and private banks.

Public banks are, in theory, a wonderful option for families. Unfortunately, the United States did not even begin a national oversight program until 2004, and the current program [<http://bloodcell.transplant.hrsa.gov/>] has not fully organized the process at this time. It can be difficult to donate, usually requiring a mother to go through testing, fill out considerable paperwork, and obtain a collection kit, all on her own prior to delivery. In addition, because the number of units in public banks remains low, if your child eventually needs his or her own banked cord blood, it may or may not still be available.

Hopefully in the future the United States will take the lead on this subject, making donation a simpler process and thereby collecting more units for public banks. As technology improves and the number of cells within a sample are able to be expanded, it may become the norm to use public banks only, since expansion of cells would allow a unit to be used for multiple patients, including the original donor or his/her family members.

At this time, choosing a public or private bank is a difficult decision to make. If you can afford to privately bank your child's blood, then by all means do so. If you are unable to shoulder such a high cost or are ethically opposed to for-profit banking, then definitely donate to a public bank. Your donation may still be available for your child's use or for another family member.

## **A Few Words on Non-US Clinics**

There are many non-US clinics around the world who claim to provide stem cell treatments for children with cerebral palsy. Some of these are complete and utter forms of quackery, while others are more legitimate. A quick search of the web will bring up several dubious sites that lack fundamental information such as the location of the facility, the doctor, the exact procedure, and so forth. Others have glitzy websites touting a wide variety of miracle cures that while enticing are entirely unrealistic.

In general, most of these clinics should be avoided entirely. The vast majority are money-making ventures with no history of results, no published research, and potentially harmful medical ramifications. One such clinic is backed by a doctor who has been repeatedly cited for malpractice and unprofessional behavior, leading to the death of one child, and serious illness in several other patients. This same clinic, along with several others, administers dubious “cocktails” which may or may not even contain legitimate stem cells and whose formulations are completely unproven. Without certification and oversight, such as is available in the United States, it is impossible to know that the treatment your child is getting is actually genuine or safe. All such programs that lack governmental oversight should be avoided entirely.

A few clinics may be more medically legitimate, but none have published any of their findings in peer-reviewed journals at this time, and none have done any studies to support their research. In addition, stem cell therapy is often coupled with intensive rehabilitative therapy and other treatments at some clinics, and it is impossible to know which of these methods has actually improved the condition of the patient without double blind studies.

One clinic in Europe performs stem cell infusions by extracting bone marrow, a very painful procedure, and infusing the stem cells from the bone marrow back into the patient through a spinal tap. While this procedure may eventually be promising, it is quite invasive and painful, and there are only several anecdotal case reports to back up its effectiveness. The cost, risks, and invasiveness of this procedure, coupled with a complete lack of scientific evidence, make it inadvisable.

While some of these treatments may ultimately be effective, until research is performed in clinics with strict governmental oversight, they are not recommended.

## **Future Steps**

Science is just beginning to understand the unbelievable power and potential of stem cells and their ability to heal the brain and regenerate neurons. Stem cells are available from a wide variety of sources, including umbilical cord blood, adult or fetal central nervous system tissue, skin, teeth, and embryos. Some of these methods are more restrictive, since the stem cells from tissue typically lack plasticity or the ability to differentiate into a wide variety of different types of cells. Others, particularly embryonic stem cells, may be restricted due to ethical concerns, though more freedom in this area of research is

expected beginning in early 2009. In addition, researchers are working on ways to reprogram skin cells and other tissue cells to avoid ethical considerations altogether.

As science progresses, however, more and more of these stem cell treatments will be possible. One study, using stem cells from fetal brain tissue to treat the devastating and fatal disorder Batten disease, is already underway for pediatric patients. [<http://www.stemcellsinc.com/clinicaltrials/clinicaltrials.html>]

An amazing future stands before us. We are beginning the first steps to what may be a curative treatment for cerebral palsy in the not too distant future.

For more information:

- ❑ Children’s Neurobiological Solutions, a not-for-profit focusing on brain regeneration [<http://www.cnsfoundation.org/site/PageServer>]
- ❑ Dr. Joanne Kurtzberg’s Program at Duke University [<http://www.cancer.duke.edu/pbmt/>]
- ❑ Overview of Stem Cells [<http://www.cnsfoundation.org/site/DocServer/stemcells.swf?docID=181>]
- ❑ Information on Cord Blood Banking [<http://bloodcell.transplant.hrsa.gov/CORD/Options/index.html>]
- ❑ Information on Questionable Practices in Non-US Clinics [<http://www.quackwatch.org/06ResearchProjects/stemcell.html>]

*Thanks to Mary Schneider for her critical insights on this topic.*

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<sup>1</sup> Information gleaned from “Mary Schneider’s Story: Stem Cells and a Mother’s Unrelenting Drive to Reverse Her Son’s Cerebral Palsy.” CNS Foundation website,

[http://www.cnsfoundation.org/site/News2?news\\_iv\\_ctrl=-1&page=NewsArticle&id=7331&security=1](http://www.cnsfoundation.org/site/News2?news_iv_ctrl=-1&page=NewsArticle&id=7331&security=1)

<sup>2</sup> A.J. Joannides and S. Chandran. “Human Embryonic Stem Cells: An Experimental and Therapeutic Resource for Neurological Disease.” *Journal of the Neurological Sciences* 2008;265:84-8.

<sup>3</sup> Erin Cline Davis, “Cord Blood: A Weapon Against Cerebral Palsy?” *Los Angeles Times*, April 7, 2008, F3.

<sup>4</sup> Joanne Kurtzberg, *et al.* “Untying the Gordian Knot: Policies, Practices, and Ethical Issues Related to Banking of Umbilical Cord Blood.” *The Journal of Clinical Investigation* 2005;115(10):2592-7.