



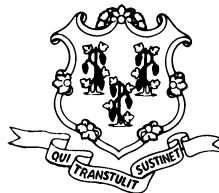
Keeping Connecticut Healthy

REPORT TO THE GENERAL ASSEMBLY

Public Act 06-77
AN ACT CONCERNING THE ESTABLISHMENT OF A
PUBLIC UMBILICAL CORD BLOOD BANK

January 5, 2007

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**Connecticut
Department of Public Health
Umbilical Cord Blood Ad Hoc Committee
Report to the Office of the Governor and the General Assembly
January 5, 2007**

Public Act No. 06-77

**AN ACT DESIGNATING THE MONTH OF NOVEMBER AS LUNG CANCER AWARENESS
MONTH AND CONCERNING THE ESTABLISHMENT OF A PUBLIC UMBILICAL CORD
BLOOD BANK.**

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**Report of the Ad Hoc Committee Concerning the Establishment of a Public
Umbilical Cord Blood Bank
January 5, 2007**

Executive Summary

Public Act 06-77¹ (the Act) established an ad hoc committee (the Committee) to examine and evaluate the feasibility of (1) establishing a public umbilical cord blood bank for the purpose of collecting and storing umbilical cord blood and placental tissue donated by maternity patients at hospitals licensed in this state, (2) entering into a multi-state public umbilical cord collaboration, and (3) developing a public-private partnership with existing umbilical cord blood banks.

Per the Act, the Commissioner of the State Department of Public Health appointed members to the Committee² and convened the inaugural meeting on July 18, 2006. During subsequent meetings held on September 11, 2006, October 30, 2006, December 5, 2006, and December 27, 2006, the Committee reviewed current federal, state and private banking programs and regulatory requirements, and received testimony from state, regional and national experts on cord blood banking practices and the clinical uses of cord blood, including a review of the use of hematopoietic cells in transplantations, and an overview of the therapeutic use of cord blood transplants in the pediatric and adult populations. The need for increased racial and ethnic diversity of the cord blood supply was reviewed at length both in terms of the Connecticut population and the national cord blood supply.

Once considered medical waste, the umbilical cord and placenta are now known to contain adult stem cells that can be used to provide a life saving stem cell transplant for patients with various malignancies. Because of its biology, the adult stem cells contained in cord blood do not have to be as closely tissue matched as do the adult stem cells obtained from the other two sources of adult stem cells, namely bone marrow and peripheral blood. This biological finding makes collection and provision of cord blood stem cells a great benefit to ethnic minorities and other patient groups who are currently unable to obtain an adequate match for a transplant using bone marrow or peripheral blood stem cells. Some of the benefits of a Cord Blood Collection and Storage Program include:

- Ease of collection.
- Less need for an identical tissue (HLA) match between donor and recipient.
- Permits ethnic and other minorities to access adult stem cells for a life saving transplant.
- Cord blood transplants are successful for both children and adults.
- Cord blood stem cells have potential for use in many emerging medical areas that require tissue growth such as “manufacture” of blood vessels for patients with heart disease.

The Committee studied cord blood banking operations in detail, including the critical components of public education, collecting cord blood units (CBU), processing and storage, and the sale of cord blood units. The economics of cord blood banking were studied and discussed in depth. The Committee identified the three major cost elements associated with establishing and operating a public bank as the capitol costs for the processing and storage facility, the labor costs associated with collecting and processing, and the costs of testing the unit for transfusion-related disease markers and donor markers of genetic diseases.

Based on its information gathering activities, the Committee agreed to the following broad guidelines in addressing the role of the State of Connecticut with respect to establishing a public umbilical cord blood bank:

¹ see Appendix A

² see Appendix B

- All birthing mothers should be informed about available public and private cord blood donation and banking options in the state.
- Connecticut should develop viable public cord blood banking options.
- Connecticut efforts should address the need for racial and ethnic diversity in and unrestricted access to public umbilical cord blood supplies.
- Connecticut does not need to develop its own public cord blood donation and banking program, and should explore partnerships with existing public and/or private cord blood banks.
- The collection, transportation, processing and storage of all umbilical cord blood from birthing mothers in Connecticut should be done in accordance with all applicable existing regulatory requirements promulgated by the federal government and by national accreditation bodies.
- Relevant components of the Connecticut health care delivery community should be fully educated about public and private cord blood banking programs and as appropriate, fully compliant with acceptable standards of practice for the collection of umbilical cord blood.

The Committee agrees that the most advantageous approach to providing for collection of CBUs within the state is through a public-private partnership between the state and a public cord blood bank that is willing to establish public collection operations within Connecticut to collect CBUs for therapeutic and research use. The chief advantage of this approach is that it provides a mechanism that will encourage collections within the state at the earliest possible time frame while avoiding the need to invest significantly in the development of the infrastructure to create a new banking facility.

There are a number of approaches to a public-private partnership. The Committee believes that the best model would be one that provides for financial support from the state to encourage collection activity coupled with an expectation of a return to the state of some or all of the sale proceeds of the CBUs collected within the state. Because the Committee was not tasked with seeking proposals for establishing collection activity, the specific terms of such an arrangement have not been determined. However, within the public-private partnership approach, the Committee recommends that the state pursue a solicitation process to seek proposals from existing banks that would contain the following elements:

1. The bank would agree to establish and operate one or more collections sites within the state to collect a targeted number of units to be determined as a part of the process.
2. The collection program would be committed to collection of CBUs that reflect the racial and ethnic and diversity of the state's citizens.
3. The bank may request an investment from the state to support collection, processing and banking.
4. Any requested state support would be offset by a proportional distribution to the state from sales of the CBUs from the partner bank, either out of the bank's general inventory or from the CBUs collected under that state program.
5. The bank would agree to set up operations within six months of the completion of the contract, provided that contracts with collection sites can be identified and negotiated within that time frame.
6. The bank would participate in the federally created Cord Blood Coordinating Center (CBCC) by listing units so that the maximum opportunity for use will be assured.
7. The bank must have a program that provides CBUs for research and will agree to provide units not suitable for therapeutic use to researchers located within Connecticut without charge.
8. The bank must be accredited as a public bank by one of the national accrediting organizations recognized by Health Resources and Services Administration (HRSA).
9. The bank must demonstrate ability to meet applicable Food and Drug Administration requirements.

It is anticipated that CBBs will be willing to establish collection sites within the state under these terms. The advantages to the state in such an arrangement are:

1. Avoid the cost of establishing its own facilities.
2. Move quickly to establish collections within the state by taking advantage of the existing programs of public cord blood banks.
3. Recognizing that some investment may be necessary to encourage collection within the state, the program would hope to recoup some or all of the investment overtime by receiving a portion of the fees charged within units are sold for therapeutic or research purposes.
4. Through its participation, Connecticut will be able to provide for collection in an equitable manner for its citizens.

The Committee considered the option whereby the state would establish a banking operation under state ownership and control but felt that this option was not desirable as it represented the highest cost and greatest risk to the state. In addition, it would take a considerable amount of time to set up. Since nongovernmental entities have already created expertise and capacity for public banking, the state could avoid the cost and risk but still accommodate the desire for public banking within the state through a public-private partnership.

Likewise, the Committee does not recommend pursuing a multi-state approach at this time. While this approach may have advantages in providing an opportunity to share the risk of investment and operations, it would require significant effort to enlist and develop such an arrangement with other states, even if the reception to such an idea were welcome. However, the state should be prepared to respond to overtures along these lines should interest be expressed as it pursues its own track, consistent with the recommendations of the Committee, if adopted.

II. INTRODUCTION

A. Charge to the Committee

Public Act 06-77, AN ACT DESIGNATING THE MONTH OF NOVEMBER AS LUNG CANCER AWARENESS MONTH AND CONCERNING THE ESTABLISHMENT OF A PUBLIC UMBILICAL CORD BLOOD BANK (The Act) was enacted on May 30, 2006 in Connecticut. Concerned about the lack of choices for public cord banking offered to birthing women in Connecticut and recognizing the therapeutic and research importance of umbilical cord blood, the General Assembly tasked the Department of Public Health, through its Commissioner, to convene an ad hoc committee (the Committee) to study the feasibility of establishing a public umbilical cord bank in Connecticut for the purpose of collecting and storing umbilical cord blood and placental tissue donated by maternity patients at hospitals licensed in the state. The Committee was also tasked with addressing the potential for a multi-state public umbilical cord partnership, and the potential for a developing a public-private partnership with existing umbilical cord blood banks. The Committee was mandated to report its findings and recommendations to the legislature and to the Office of the Governor by January 5, 2007.

B. Overview of Developments in Cord Blood Banking

According to the National Institute of Medicine³, stem cells are a primitive cell type that are found in all types of animals, and that are different from all other types of cells. All stem cells share three common traits: they are capable of dividing and of self-renewal for periods of time; they are unspecialized; and they have the ability to develop into specialized cell types through the process of differentiation.

The National Institute of Medicine classifies three types of stem cells based on their ability to differentiate:

- Totipotent cells - capable of giving rise to all the different types of cells within the body as well gestational support structures like the placenta. Examples are the fertilized egg (zygote) and the individual cells of the cleaving zygote prior to the fifth cellular division (blastomeres).
- Pluripotent cells - capable of giving rise to all the different types of cells within the body but not the gestational support structures. An example is embryonic stem cells.
- Multipotent cells - capable of giving rise to a limited number of different cell types. Examples are blood stem cells and neural stem cells. Multipotent stem cells are also called "adult stem cells" or "tissue-specific stem cells".

Due to the diagnostic and therapeutic potential of pluripotent stem cells and the current federal restrictions on funding research with non-federally approved pluripotent, human embryonic stem cells, the State of Connecticut funds and administers its own Stem Cell Research Program. Additional information on this program can be found on the state Department of Public Health's web site at <http://www.dph.state.ct.us/stemcell/index.htm>.

This report focuses on a specific multipotent stem cell, the Hematopoietic Progenitor Cell (HPC), and more specifically, an adult HPC obtained from umbilical cord blood (UCB). To reiterate, even though these are cord blood cells from a newborn, biologically they are classified as adult HPCs.

³ Institute of Medicine: Cord Blood: Establishing a National Hematopoietic Stem Cell Bank Program, (National Academies Press, 2005)

HPCs can differentiate into the vast array of normal blood cells, including the cellular components comprising the blood and the immune system. HPCs can be obtained from a number of different sources, including bone marrow, peripheral blood, and from UCB collected from the placentas of recently delivered infants. HPCs have been transplanted to treat a number of blood diseases, selected metabolic disorders and immunodeficiencies, and sickle cell anemia.

As early as 1982, umbilical cord blood was identified as containing hematopoietic progenitor cells suitable for transplants⁴. HPCs obtained from umbilical cord blood appear to have some advantage over HPCs obtained from bone marrow or donor blood. According to the National Institute of Health, cord blood HPCs appear less mature than those from other sources, so their transplantation results in a lower risk of graft-versus-host disease. Cord blood HPCs are readily available, and collection is painless and safe⁵.

The first UCB transplant in the United States was reported in 1988. Since then, for the thousands of patients each year needing a bone marrow transplant but lacking an appropriate match, or for those patients too sick to wait for a lengthy screening, identification and harvesting program, cord blood derived HPCs have offered a viable option for life saving therapies. According to the National Cord Blood Program⁶, there have been more than 6,000 cord blood transplants throughout the world to date. Given the therapeutic results, there appears to be an ever-increasing number of therapeutic applications for cord blood transplant treatment of thousands of patients.

III. USE OF HEMATOPOIETIC CELLS IN TRANSPLANTATION

A. Overview

Diseases occurring within the blood or hematopoietic system have long plagued mankind. Skeletal remains from the fourth century showed the terminal stages of multiple myeloma⁷, a rare form of bone marrow cancer. The recognition of acute myeloid leukemia (AML) as a discrete, clinical entity came in 1827 by the French surgeon Alfred Velpeau. The term leukemia was coined by Rudolf Virchow, in 1847⁸.

Various regimens for treating leukemia were attempted early on but it was not until World War I that chemicals known as nitrogen mustards – including mustard gas, were noted to have the ability to destroy blood-producing bone marrow cells. Thus, it became possible to destroy diseased blood tissue in conditions such as leukemia. While nitrogen mustards and other compounds were very potent at eliminating diseased blood tissue, unfortunately they also destroyed healthy blood tissue. Under-treating patients left them with residual disease that quickly relapsed. Overly aggressive chemotherapy, however, left patients anemic, bleeding-prone, and susceptible to infections provided they survived the treatment at all. The ability to replace blood-producing tissues was thus seen as having strong therapeutic potential. Indeed, HPC transplant had the potential to offer a true lasting cure for the patient suffering from hematological malignancies and other blood diseases.

In 1957, E. Donnall Thomas reported on his laboratory's early attempts at a procedure to infuse bone marrow cells from one person into another (allogeneic transplantation)⁹. By the 1980's, bone marrow transplantation had become an effective therapy offered in many medical centers

⁴ Cellular Characteristics of Cord Blood and Cord Blood Transplantation, Broxmeyer, H.E., 1988

⁵ <http://www.cordblooddonor.org/>

⁶ <http://www.nationalcordbloodprogram.org/>

⁷ Greaves M. *Cancer: The Evolutionary Legacy* (Oxford University Press, Oxford, 2000).

⁸ Henderson ES. in *Leukemia* (eds. Henderson, E. S., Lister, T. A. & Greaves, M. F.) 1-7 (W.B.Saunders, Philadelphia, 1996).

⁹ E.D. Thomas, H.L. Lochte, Jr., W.C. Lu and J.W. Ferrebee, *N. Engl. J. Med.* 257, 491-496 (1957).

worldwide, and in 1990, Dr. E. Donnall Thomas received the Nobel Prize for pioneering the field of bone marrow transplantation¹⁰.

Today, blood stem cell transplantation is offered as a therapeutic maneuver for a wide variety of hematologic, metabolic and genetic diseases¹¹. It was initially thought that only bone marrow could provide the HPC needed for transplantation. We have now learned that HPC can be recovered from other sources as well. A second source is from peripheral blood. This requires that the donor be stimulated with a drug (G-CSF – a white cell stimulating factor) that stimulates the bone marrow to release its stem cells into the peripheral blood where they can be collected by an automated blood collection device known as an apheresis machine. Such machines are used daily at blood centers worldwide to collect blood products such as platelets for transfusion. More recently, it was discovered that blood from the umbilical cord and placenta contain valuable HPCs. Once viewed as postpartum medical waste, umbilical cord or placental blood is now highly valued for its HPCs. Indeed, as will be discussed below, cord blood can be used to transplant adults as well as children. Thus, we now recognize three primary sources of adult HPCs for transplantation:

- bone marrow
- G-CSF-stimulated peripheral blood
- cord blood

While it has improved the lives of thousands of people, many more still have been unable to take advantage of this life-extending therapy for a variety of reasons. The inability to locate a suitably matched tissue donor stands out above the rest. While there are many genes that are key to the immune recognition process involved in matching, three genes are known to be of central importance. They are HLA-A, HLA-B, and HLA-DR. These genes are inherited by each of us from our parents. We have six copies of HLA genes in total, two HLA-A genes, two HLA-B's, and two more HLA-DR's, one of each from our mother and one of each from our father. This is why the best match is often called a 6/6 ("six out of six") match. Today, using the most sophisticated methods of HLA typing, additional so-called minor antigen variations have been identified. Indeed, now the best match is viewed as a 10 out of 10 antigen match. The better the match, the more likely it is that the transplant or graft will not fail. The greatest chances for finding a good match are with one's own siblings, especially if one has an identical twin, since identical twins share the exact same genetic makeup.

Tissue matching refers to a genetic match between the tissue of the patient and that of the donor, so that the patient's body does not reject the needed transplant as being "foreign", thus leading to tissue rejection, i.e., graft failure. There is another complication that may occur as a consequence of tissue rejection, known as "graft versus host disease" or GvHD. In this potentially lethal condition, immune cells present in the transplanted tissue attack the patient's body. In a sense, the graft actually rejects the patient.

The crux of the problem with HLA matching is that the HLA antigen system is incredibly diverse (pleomorphic). Due to a very large number of variations of these antigens known as alleles, enormous numbers of different combinations of these important antigens exist. This explains why the HLA antigen system is so complex. Various ethnic groups tend to share similar alleles. However intermarriages and the mobility of the earth's population have led to a global spreading of the HLA genes. Thus, if someone from a particular ethnic group does not have many blood relatives, their chances of finding a 6/6 match are slim unless a donor registry is large and diverse enough to include people from all over the globe where their ancestors may have migrated or intermarried.

For the reasons described above, it is possible that a match might be found in another more distantly related family member or even a total stranger. Generally, however, a good 6/6 (or

¹⁰ http://nobelprize.org/nobel_prizes/medicine/laureates/1990/thomas-lecture.pdf

¹¹ http://www.leukemia-lymphoma.org/hm_lls

10/10) HLA antigen match becomes less likely the more distantly related the donor is to the patient. Of course, a person does not have to be related to the patient at all. While a stranger can also be a match, there is simply a lower probability of such a match occurring with an unrelated donor, compared to a close biological family member. This is why national and international bone marrow donor databases such as that maintained by the National Marrow Donor Program are so important¹². Thus, a donor on a registry in Spain, might just be a match for a patient in Connecticut- or vice versa.

Despite such registries, however, vast donor shortfalls remain. As many as 2/3 of all patients needing such a transplant will be unable to locate a suitable donor and this figure may be even worse among certain ethnic groups¹³. Alternative sources for transplantable blood-forming tissue have been sought. One such resource is umbilical cord blood, also known as placental blood.

B. Overview of the Therapeutic Use of Cord Blood

1. Umbilical Cord Blood Transplants in Pediatrics:

Umbilical cord blood (UCB) has been used as an alternative source of hematopoietic stem cells (HSC) for transplantation in the pediatric setting for over a decade. As discussed, HPC transplantation has been used effectively in the setting of both malignant and nonmalignant diseases. Umbilical cord blood has a large number of blood stem cells¹⁴. At birth, this blood is discarded along with the umbilical cord and the placenta. Recognizing that this discarded material had the potential for great clinical importance, studies began to characterize its blood-forming activity and applicability to clinical transplantation.

The first successful blood-forming tissue transplant using umbilical cord blood as a cellular source, i.e., a cord blood transplant, was performed by Dr. Eliane Gluckman of the Hospital Saint-Louis of Paris in 1989¹⁵. The patient, a child suffering from a rare genetic blood disease Fanconi anemia, is today a young man alive and well almost twenty years post-transplant. He is being followed by Dr. Joanne Kurtzberg of Duke University. Following an invitation, Dr. Kurtzberg came to Connecticut to address the Cord Blood Committee and she provided valuable input into the Committee's charges.

UCB Transplant Survival is influenced by several variables which include:

- donor type (related family member versus unrelated donor registry member)
- primary disease (malignant vs. nonmalignant disease)
- HLA match (see above)
- number of stem cells (dose) infused¹⁶

Data have shown that unrelated UCB transplants in children can be successful with reported disease free survival ranging from 29% to 85%¹⁷. For adult bone marrow transplantation, generally, the closer the HLA antigen match between donor and recipient the better is the transplant outcome. However, UCB transplantation has been shown to be successful even when the patient and cord blood donor are mismatched at 2 antigens (i.e. a 4/6 match). If the biology will allow a successful UCB transplant even with a mismatch at 2 of the 6 HLA antigen sites, this

¹² <http://www.marrow.org/>

¹³ see Lensch and Daley "Scientific and clinical opportunities for modeling blood disorders with embryonic stem cells" *Blood*. 2006 Apr 1;107(7):2605-12 and references therein.

¹⁴ Lensch and Daley "Origins of mammalian hematopoiesis: in vivo paradigms and in vitro models" *Curr Top Dev Biol*. 2004;60:127-96.

¹⁵ Gluckman E. et al., "Hematopoietic reconstitution in a patient with Fanconi's anemia by means of umbilical-cord blood from an HLA-identical sibling" *N Engl J Med*. 1989 Oct 26;321(17):1174-8.

¹⁶ Brunstein CG, Wagner JE. Umbilical cord blood transplantation and banking. *Ann Rev Med* 2006; 57:03-17.

¹⁷ Balen KK. New trends in umbilical cord blood transplantation. *Blood* 2005; 3786-92.

would allow more donors to be acceptable and available as a match for patients in need of a transplant. Such a scenario would expand the donor pool considerably and increase the number of recipients who could receive a life saving UCB transplant. It has been estimated that approximately 80% of pediatric patients who undergo an unrelated UCB transplant receive a mismatched UCB unit¹⁶.

It is important to remember that randomized clinical trials comparing the outcome of unrelated bone marrow transplantation (BMT) versus unrelated UCB transplantation in pediatric patients with acute leukemia has not been undertaken. However, several retrospective comparative analyses between unrelated BMT and UCB transplants have been reported^{16,17,18}. While engraftment, particularly platelet and lymphoid (lymphocyte) engraftment, is delayed in patients receiving UCB transplants, the incidence of severe graft versus host disease is low and the endpoint of relapse rate and survival appear comparable. This suggests that for UCB transplants, some level of mismatch can be acceptable and still result in a good outcome for the patient. If extrapolated, this could mean that the more units of UCB stored, the more likely that someone – adult or child- would be able to find a match or a partial mismatch among the stored units of cord blood and thus receive a life-saving HPC transplant that otherwise would not be available. Importantly, whether the good outcome seen with a mismatched UCB transplant for a child will have a similar good outcome if the same degree of mismatched cord bloods were transplanted into an adult is not yet known.

2. Umbilical Cord Blood Transplants in Adults:

Progress of UCB transplants in adults has been slower than in children secondary to the low cell dose available with a cord blood unit, in comparison to other sources of stem cells and the likelihood of an HLA antigen mismatch. As adults are bigger, the same dose of UCB stem cells represents a higher dose in a small child versus giving that same dose to a physically larger adult. Since the dose of HPC is one of the important variables for determining a successful outcome post-transplantation, this is not a trivial issue. The most frequent indications for UCB transplantation in the adult tends to be acute and chronic leukemia^{19,20}. There has been considerable discrepancy in outcome data looking at single unit UCB transplants in adults, with survivals ranging from 19% to 74%^{16,17,21,22}.

A recent review of three large studies highlights the feasibility of UCB transplants and differences between the sources of stem cells²⁰. The conclusions from the three retrospective analyses were slightly varied. The differences observed among these studies reflect many variables. Prospective clinical studies will be necessary to truly answer the question.

The studies did show, however, that engraftment after UCB transplant could be achieved with a decreased incidence of both acute and chronic graft versus host disease (GvHD). However, the time to UCB engraftment was delayed in comparison with unrelated BMT. This tends to result in early transplant-related mortality, mostly as a result of infection. Generally, the longer the time to engraftment the longer the recipient is vulnerable to potentially life-threatening infections. Infected transplant recipients who have not engrafted, are a high risk of death from bacterial infections, even if they are treated with antibiotics since they have a compromised immune system. To overcome the limitations of low dose in adult patients and delayed engraftment, recent investigation has concentrated on evaluating the use of infusing two cord blood units in adults either as a pool or sequentially. Preliminary studies have shown that this double UCB approach is feasible with engraftment and survival rates, based on historical data, appearing to be more

¹⁸ Rocha V, Cornish J, Sievers EL, et al. Comparison of outcomes of unrelated bone marrow and umbilical cord blood transplants in children with acute leukemia. *Blood* 2001;2962-71.

¹⁹ Schoemans H, Theunissen K, Maertens J, et al. Adult umbilical cord blood transplantation : a comprehensive review. *Bone Marrow Transplant* 2006;38:83-93.

²⁰ Ballen, KK, Haley N, Kurtzberg J, et al. Outcome of 122 diverse adult and pediatric cord blood transplant recipients from a large cord blood bank. *Transfusion* 2006;46:2063-70.

favorable than that reported previously for bone marrow or peripheral blood transplants^{16,17,19}. Curiously, even though two cord blood units are infused, only one “wins” and engrafts. There is currently no way to determine which cord will ultimately “win out”.

IV. CORD BLOOD BANKING

A. Umbilical Cord Blood Banks

The NIH defines a cord blood bank as a center whose central mission is to maintain a supply of cord blood for therapeutic use in transplantation. Public banks collect and store unrelated cord blood units donated altruistically for research or transplantation. Private cord blood banks store cord blood for autologous or family use only, and typically charge a fee for the collection, processing and storage of the cord blood. The first public umbilical cord blood bank was established in 1993 as the New York Blood Center’s (NYBC) National Cord Blood Program. According to its web site⁶, more than 33,000 mothers have donated their baby’s cord blood to the NYBC National Cord Blood Program. Donors come from all ethnic backgrounds: 20% are African-American, 21% Hispanic-American, 8% Asian-American and 48% Caucasian. The program has provided cord blood units for transplantation to over 2,000 recipients to date--approximately one third of all cord blood transplants from unrelated donors worldwide. Most recipients have been affected by leukemia, lymphoma, severe aplastic anemia and other lethal diseases of the blood – both malignant and non-malignant (i.e., sickle cell disease).

One of the first private cord blood banks and currently one of the largest is ViaCord, a ViaCell Company established in 1993 and located in Cambridge, Massachusetts. According to their web site²¹, ViaCord has banked the cord blood of more than 80,000 families globally, and have used nearly two dozen of their banked units for transplantation. The costs include \$1,800 for processing, and an annual storage fee of \$125.

In the thirteen years since the establishment of these public and private cord banks, more than 40 different public, private and hybrid banks have been formed in the United States alone. A Google search under umbilical cord blood banks results in about 473,000 hits²². This proliferation of private and public cord blood banks in the absence of standardized regulatory requirements led to questions from consumers, practitioners and regulators concerning the collection, processing and storage of units. The absence of a national cord blood bank also led to a number of state-specific legislative initiatives²³, including a legislative-mandated feasibility study here in Connecticut.

Recognizing the need for and value of a national cord blood bank, the Congress asked the Institute of Medicine to review the options for such a system and to make recommendations for establishing such a program. In 2005, the NIH recommended the establishment of a national cord blood bank in its publication of CORD BLOOD: ESTABLISHING A NATIONAL HEMATOPOIETIC STEM CELL BANK PROGRAM³. Recommendations in the report included the establishment of a national Cord Blood Policy Board, a competitive bid process to solicit proposals for running a national program, and a goal of collecting at least 150,000 viable units of cord blood. In December 2005, President Bush signed into law the STEM CELL THERAPEUTIC AND RESEARCH ACT²⁵, authorizing \$79 million in new federal funding for the collection of

²¹ <http://www.viacord.com/>

²² <http://www.google.com/search?hl=en&q=umbilical+cord+blood+banks>

²³ see Appendix C, from the Cord Blood Registry, website at <http://www.cordblood.com>

²⁴ see Appendix D

umbilical cord blood units from ethnically diverse donors and the establishment of a national public cord blood bank network.

B. Banking Operations

The business of cord blood banking for public use is composed of four elements, public education, collection, processing and storage and sale of the unit.

1. Public Education

In order to effectively recruit donors for cord blood donation, education must be a key element in cord blood donor program. Education would focus on three audiences; practitioners, patient donors, and hospitals. Printed materials as well as a toll free number for information are necessary for outreach education and support.

Obstetricians, gynecologists, pediatricians and nurses would need to be educated on the cord blood donor program. A protocol would be presented to the obstetricians on when they would introduce cord blood donation, during the pregnancy. Hospital administrations and labor and delivery staff would need to be educated on the protocol for collecting and transferring donated cord blood to the state bank.

Practitioners are the primary source for recruiting donors. Most patients learn about cord blood thought their prenatal visits. Usually around the 16th week of pregnancy, a pamphlet is included in the patient's prenatal information folder, explaining cord blood stem cells, their transplant applications and the opportunity to privately bank or publicly donate. The patient is then asked to review the materials and to let the physician know of their decision, usually by 28-30 weeks for public donation. It is at this time the nursing or medical staff follows up with the patient regarding questions they may have on cord blood collection and reiterate the differences between private collection and public donation.

An effective educational outreach program is critical for successfully recruiting patients and implementing the established protocols for collection, transport and storage of cord blood units. Understanding the therapeutic applications for cord blood stem cells is important for patients, in order to make an educated and informed decision.

2. Collection

Cord blood units must be collected at the time of birth, immediately after delivery. Prior to collection, and before the onset of labor, the donating mother is provided with information regarding donation and consent is obtained, preferably during the third trimester of pregnancy. Once consent is obtained a family and maternal risk screening is performed. This assessment of the health of the mother and the family history is intended to screen out any units that may be unsuitable for donation due to maternal high risk behaviors such as drug use, infection with transfusion transmitted diseases or a family history of diseases that could be transmitted genetically to the UCB recipient.

Once a unit has been qualified for a harvest, collection follows immediately after birth in the delivery suite. The donation can be obtained either in-utero (with the placenta still attached to the uterine wall) or ex-utero (following expulsion of the placenta) depending on the process preferred by the collecting site and the cord blood bank. The unit is initially qualified for storage based upon the volume of cord blood collected, as this is directly proportional to the amount of HPCs collected. If sufficient cells are assumed present from the collection, the unit is transported to the cord blood bank for processing.

3. Processing and Storage

Before a unit is finally qualified for banking, two additional screening steps are taken at the processing site. First, the unit is tested for infectious disease markers to determine whether any additional blood-borne diseases could be transmitted by the unit. In addition, the total nucleated cell count of the unit is established to determine whether it's of sufficient size for therapeutic use. Assuming these tests indicate the desired results, the unit is processed further, including steps for plasma and red cell depletion, mixed with a cryopreservative to protect the cells during frozen storage, and put through a process of controlled rate freezing so that the unit may be stored at a temperature no warmer than -150° Celsius in liquid nitrogen. Appropriate labeling is applied to the storage bag prior to freezing.

4. Sale of Cord Blood Units

Cord blood units are made available to transplant centers by establishing a registry of available units either directly through the bank or via listing the units on a listing registry such as that maintained by the National Marrow Donor Program. When a physician has a patient who may need an hematopoietic stem cell transplant, the physician will search the cord blood registry to determine whether a cord blood unit that is a close match for the patient is available. Matching is determined by comparing the HLA type of the patient with that of the available cord blood unit. If a unit is acceptable, the transplant center will order the unit. The unit is shipped to the transplant center in a liquid nitrogen shipper so that it arrives at the transplant center, as it was stored, in a fully frozen state. After thawing and preparing the unit for infusion, typically by washing out the cryopreservative, the unit is infused intravenously by the transplant center medical team in the same manner as one performs a blood transfusion.

V. ECONOMICS OF CORD BLOOD BANKING

Cord blood banking requires a significant initial investment of capital to develop the needed infrastructure and to build an inventory sufficiently large to develop a sustaining income stream.

A. Demand for Unrelated Transplants

NMDP has estimated that approximately 14,600 Americans will need an unrelated transplant each year. Of that number, approximately 2,500 actually receive a transplant. While there are many reasons for this discrepancy, the most significant barriers to transplant include lack of adequate insurance, other financial issues, psycho-social issues, bias against treatment by treating physicians and a lack of an appropriate cell source. The last two elements, bias against treatment and lack of cell source, are addressed in part by an expanding inventory of publicly available cord blood.

The bias against treatment includes concern that the treatment related mortality is high and the complications from even successful treatment are not acceptable. As discussed earlier, cord blood has the promise of addressing both of these issues to some degree.

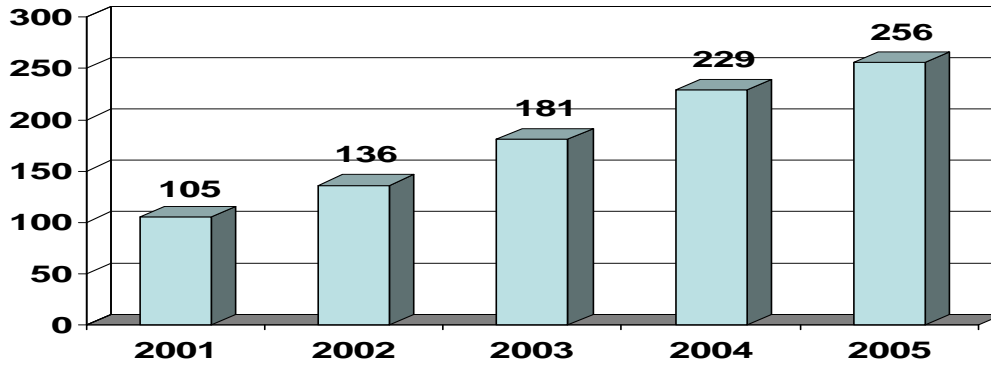
Cord blood also addresses the lack of an available cell source, serving to complement the adult registries by expanding access for those unable to find a suitable matched donor. NMDP experience shows that African Americans make up only 7% of the adult donor recipients by almost 15% of the cord blood recipients.

Other factors that bear upon the increase in transplantation include capacity limitations at transplant hospitals, availability of trained physicians and support staff, and competition for other demands on hospital space. It is unlikely that while transplants will continue to climb, the demand will increase at a dramatic rate given these other factors.

B. Supply of Public Cord Blood Units

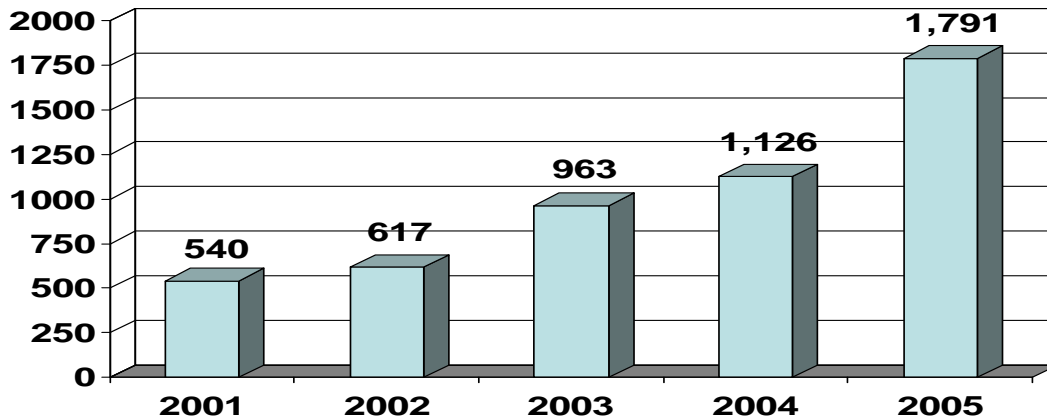
The World Marrow Donor Association (WMDA) collects data from most of the public cord blood banks regarding sale and use of CBUs. At the end of 2005 the worldwide supply of CBUs was 256,000²⁶.

Number of cord blood units available for transplantation worldwide (x thousand)
World Marrow Donor Association



In 2005, participating banks reported that 1,791 units were sold for transplantation world wide, of which 813 were used within the United States. In 2004, 519 units were used in the United States.

Number of cord blood units provided for unrelated transplantation worldwide
World Marrow Donor Association



²⁶ World Marrow Donors Association, Cord Blood Banks/Registries Annual Report 2005

The rate of utilization was less than 1% in each of the last three years, even as the demand has increased significantly during that same period because of the continuing growth in supply.

Given that there are other barriers to cell source, it is likely that while demand continues to grow over the next few years, the total supply will also increase at a near proportional rate so that the rate of utilization will not change materially. While the growth in supply is necessary to improve chances of finding an optimal match for an ever greater portion of the population, this has implications for the short term financial success of public cord blood banking.

C. Cost of Banking²⁷

While many women are willing to donate their child's cord blood unit for public use, because of the stringent quality standards that must be met, only three out of every ten units originally identified for possible collection are ultimately put into the bank. As described above, the screening process will eliminate units for a variety of reasons. Because HLA matching is a critical factor in determining whether a unit is appropriate for therapeutic use for a given patient, a significant number of units must be stored in order to assure an adequate availability of the cord blood unit for an individual patient. The Institute of Medicine has estimated that there is a need for at least an additional 100,000 units in a national inventory together with an adult donor registry of 2,267,000 adults to provide a match for at least 90% of the United States population at the minimally accepted match level. However, because a higher HLA match (5/6, 6/6 10/10) is always desirable for a patient, and because the importance of matching is just becoming apparent, an inventory size of in excess of 300,000 CB units or more may be desirable.

Because this inventory must be on hand in order to be useful for therapeutic purposes, cord blood banking requires significant up-front investment in capital and labor in order to develop sufficient inventory to provide units for therapeutic use.

Cord blood banking requires a significant initial investment of capital to develop the needed infrastructure and to build an inventory sufficiently large to develop a sustaining income stream. The three major cost elements of cord blood banking are:

- capital costs for the processing and storage facility
- labor costs associated with collecting and processing the unit
- costs of testing of the unit for transfusion transmitted disease markers and donor markers of genetic diseases .

The facility costs include the space necessary to house the processing lab and storage facilities, the lab processing equipment, the cryopreservation freezers, and miscellaneous equipment including the liquid nitrogen shippers used in the transportation of a cord blood unit to the transplant center.

Labor costs include the cost of recruitment of mothers for donation, the costs collection of the unit itself, although this financial cost is often donated by obstetrical staff, and the labor cost for the UCB processing stage.

Finally, testing costs, particularly those of HLA, viral marker and genetic disease testing, can be significant. The direct costs associated with the screening, collection, and storage of the unit and all necessary testing, typically are between \$1,000 and \$1,700 per unit actually banked.

The National Marrow Donor Program has estimated that approximately \$10 million is necessary to develop and store sufficient units in the cord blood bank for the bank as a fiscal entity to expect to reach a point where the bank would break even on an annual cash flow basis. This assumes a modest banking level of 2,000 units per year, which would require collections of 6,500 per year.

²⁷ Much of the content of the following section was adapted from the report by the Institute of Medicine: Cord Blood: Establishing a National Hematopoietic Stem Cell Bank Program, (National Academies Press, 2005)

The ability to obtain a return on this investment is unclear because of the demand for cord blood is still emerging. The two most significant variables in determining profitability for cord blood banking are the number of units stored and the number of units sold. Attached to this report is a summary pro-forma that shows the typical program.²⁸

To demonstrate the sensitivity of the key variables, the typical program shown on the attachment will require a \$10 million investment over the first five years to bank 2,000 units per year. Below is a grid demonstrating the impact that increased collections and percentage of inventory sold has on the investment requirement. As noted, current sales appear to support a sales rate of 1%.

		annual % of inventory sold		
		0.50%	1.00%	1.50%
Number of units banked annually and subsidy required	2000 units			
	breakeven	never	7yrs	5yrs
	investment required		\$10m	\$5.8m
	3000 units			
	breakeven	15+years	7yrs	5yrs
	investment required	\$25m+	\$13m	\$8m
	4000 units			
	breakeven	never	7yrs	5yrs
	investment required		\$16m	\$10m

VI. FEDERAL INVOLVEMENT IN CORD BLOOD BANKING

A. Development of a National Program²⁹

In 2005 Congress passed and the president signed into law the Stem Cell Therapeutic and Research Act of 2005. This act created the C.W. Bill Young Cell Transplantation Program (Program), a totally revamped system for delivering hematopoietic stem cell transplantation options to the U.S. public. Prior to this legislation, federal support was directed through previous legislation that created the National Bone Marrow Donor Registry (Registry). That legislation focused on creating a registry of adult volunteers, willing to donate marrow or peripheral stem cells and was operated by the National Marrow Donor Program (NMDP). More recently, NMDP, through the operation of the registry, expanded it to include a listing of publicly available cord blood units from most of the banks in the United States.

The new legislation is sweeping in its scope, effectively reauthorizing the Registry but expanding the federal role in a number of ways. First, it provides funding to create a National Cord Blood Inventory (NCBI), which is to be established by contracts awarded to individual cord blood banks that are charged with responsibility to begin collection of 150,000 new, high-quality cord blood

²⁸ see Appendix E

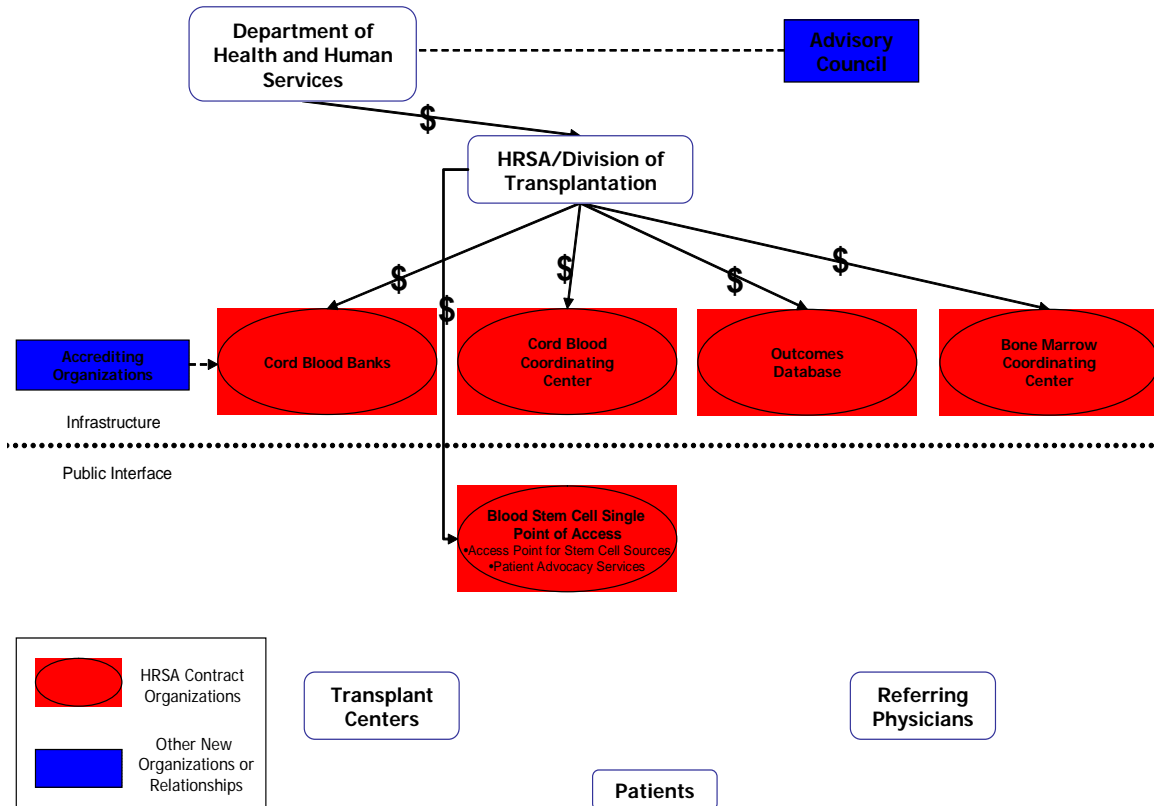
²⁹ Some of the content of the following section was adapted from the article by Dennis L. Confer, MD, The C.W. Bill Young Cell Transplantation Program and the NMDP, Newsletter, Vol. 12, Issue 2, Dec 2006, Center for International Blood & Marrow Transplant

units (CBU). Next, the Program calls for the creation of a National Cord Blood Coordinating Center (CBCC). The purpose of the CBCC is to provide a single registry for listing, searching and distribution of the CBU inventories of NCBI banks (both NCBI and non-NCBI units) as well as the inventories of other qualified banks. The third feature of the Program is the establishment of the Bone Marrow Coordinating Center (BMCC) which will oversee adult donor recruitment, donor search, product collection and product distribution activities of the adult donor registry. The public interface to these coordinating centers will be provided through the fourth element of the Program, the Office of Patient Advocacy/Single Point of Access (OPA/SPA). The OPA/SPA was created to provide the public interface with the coordinating centers to assure that there is a single point of access for all cell sources and that patients would have access to additional services to assist them in the transplantation process.

Finally, the legislation called for a Stem Cell Transplant Outcomes Database (SCTOD). The charge to the SCTOD is to collect and make available outcomes data on all stem cell transplants done within the United States, and certain transplants outside of the US as well. The outcomes data are to be made available for specifically defined research by the operator of the SCTOD as well as to the scientific community at large.

Federal oversight of the Program rests with the Department of Transplantation in the Health Resources and Services Administration (HRSA). HRSA is a federal agency within the Department of Health and Human Services dedicated to improving health care access in the U.S. In HRSA's own words, "HRSA is the nation's access agency – improving health and saving lives by making sure the right services are available in the right places at the right time." HRSA has visually depicted the program as follows.

Stem Cell Therapeutic and Research Act of 2005: Program Structure



In October, 2006, HRSA announced the first round of NCBI funding, selecting six cord blood banks as charter members of the NCBI. They are: Carolinas Cord Blood Bank at Duke University Medical Center, MD Anderson Cord Blood Bank, Milstein National Cord Blood Bank Program at the New York Blood Center, Puget Sound Blood Center, StemCyte, Inc., and the University of Colorado Cord Blood Bank.

Contracts to operate the CBCC, BMCC and OPA/SPA were awarded to the NMDP. Because NMDP received these latter three contracts, much of the complexity inherent in the Program will be shielded from the public and from the transplant community. The CIBMTR was awarded the SCTOD. The CIBMTR is a partnership of the Medical College of Wisconsin and the NMDP.

Because HRSA's mission includes a strong emphasis on access, the new contracts with NMDP include many requirements related to improving access to transplantation therapies. Some of the most interesting requirements relate to enhancing the information supplied to patients, their families and the public. For example, NMDP must develop software that allows patients, families and the public at large to conduct searches of the adult donor and CBU registries. This is envisioned as a public web site where any individual with HLA data can obtain information about the potential for matching adult donors and CBU. While this service clearly provides patients with greater access to health-related information, it also creates obligations to ensure that the information is accurate, properly represented and accompanied with important disclaimers. For example, the public search report cannot fully anticipate the transplant center's eligibility rules or HLA matching requirements.

The new contracts also require that patients are periodically updated about the status of their donor/CBU search that is being managed by a transplant center. If the search is interrupted or cancelled, the contractor must notify the patient. These requirements will be difficult to implement in a "fool-proof" manner, but will work best with solid collaboration between NMDP and the transplant centers. Additional contract requirements relate to increasing transplantation activity, improving efficiency and developing performance measures.

B. Food and Drug Administration Regulation of Human Cell, Tissue, and Cellular and Tissue-Based Products

The Food and Drug Administration (FDA) first announced its proposed approach to the regulation of cell and tissue products in 1997 (FDA, 2004b). Since then, FDA has released a series of guidelines and regulations that provide a regulatory framework for the use of human cell, tissue, cellular, and tissue-based products (HCT/Ps). FDA published a set of proposed regulations in January 2001. This proposal introduced FDA's concept of current good tissue practices around three major goals: 1) preventing the unwitting use of contaminated tissues with the potential for transmitting infectious disease; 2) preventing improper handling or processing that might contaminate or damage tissue; and 3) ensuring that clinical safety and effectiveness is demonstrated for most tissues that are highly processed, used for non-homologous purposes, or combined with no tissue components or that have systemic effects on the human body (Gee and Biol, 1999; FDA, 2004a;2004b).

A final rule that put into place provisions that require establishments that work with cells and tissues for transplantation to register with FDA and list their products was published January 19, 2001. Additional regulations incorporating comments on the draft regulations received from the public were incorporated into the final rule which was released in two parts in 2004. They became effective in May 2005. The main focus is to ensure that all processing of HCT/Ps is controllable and accountable during the collection and processing of the units. The FDA proposal contains several exceptions involving minimally manipulated cells, including cells that are harvested for autologous or reproductive use but that are not processed and stored for commercial use, such as for the directed donation of cord blood units or ova for infertility (FDA, 2004b). FDA has assumed a role in HCT/P regulation because the manufacturing and transplantation of these products often involves interstate commerce. For example, cord blood units can be collected in one state, processed and stored in another, and transplanted in a patient in yet a third state.

Despite these regulations, however, FDA has not yet licensed cord blood as a standard therapy. Its most recent discussion of the topic was at the FDA Biological Response Modifiers Advisory Committee (BRMAC) meeting on February 27, 2003, during which the committee was asked to discuss:

1. factors that FDA should consider in determining the safety and efficacy of the use of cord blood transplantation for hematopoietic reconstitution,
2. the role of the CD34+ cell count in the selection of cord blood units, and
3. other measures of quality that should be considered (BRMAC, 2003).

On the basis of data provided to BRMAC by Pablo Rubinstein and Cladd Stevens of the NYBC, it found that older recipients as opposed to children have poorer outcomes because of their higher body weights. They also noted that although the only true measure of the success of an HPC transplant is hematopoietic reconstitution in a myeloablated recipient, the CD34+ cell content is an accurate predictor of engraftment success (BRMAC, 2003).

BRMAC did agree that cord blood transplantation is an accepted approach for the treatment of a variety of diseases and that the use of bone marrow or cord blood for the treatment of particular diseases should be made on the basis of medical judgment and availability. Finally, BRMAC agreed that the general outcome parameters recommended for clinical trials of other types of HPC transplantation are suitable for clinical trials of cord blood transplantation (BRMAC, 2003).

As of January 2004, all public and private cord blood banks were required to register with FDA. However, licensure of cord blood units is still pending. Despite of the lack of licensure, many of the public cord blood banks have voluntarily submitted investigational new drug applications (INDs) to the FDA and have actively collected clinical data to be used to support the development of product standards and licensure. The IND process requires a full application explaining the study goals and methods of a new therapy. The investigator must also file periodic reports on the progress of the trial and immediate reports upon occurrence of unexpected adverse events. This allows FDA supervision in the absence of any other control, and provides a feedback mechanism which is not present in the accreditation process.

VII. COMMITTEE DELIBERATIONS

The Act specified the credentials of potential Committee members, including researchers from public and private institutions of higher learning in the state, one representative of an educational and business support network organization for bioscience in the state, one individual who is a member in good standing of the American Association of Blood Banks, with expertise in umbilical cord blood banking and the Food and Drug Administration's federal safety standards for umbilical cord blood banks, one individual with multiple years of experience in establishing, executing and administering an umbilical cord blood registry, and one member of the Connecticut's Stem Cell Research Advisory Committee. The Commissioner of Public Health serves as chairperson of the Committee. All members of the Committee were appointed in compliance with statutory requirements and deadlines.

The inaugural meeting of the Committee was held on July 18, 2006. Committee members received descriptive information regarding the Act, and an overview of current umbilical cord blood banking and use. The Committee agreed to address both the philosophy of the state with respect to umbilical cord blood banking and the feasibility of establishing public banking in Connecticut. The Committee requested additional information on current federal, state and private banking programs and regulatory requirements.

At its September 11, 2006 meeting, the Committee reviewed federal, state and private initiatives and regulatory schema related to blood banking. The Committee reviewed the impact of the

Stem Cell Therapeutic and Research Act of 2005, and the efforts of a number of public and private blood banks. With respect to the philosophical approach to the question of establishing public banking opportunities in Connecticut, the Committee agreed it was important to move forward in order to contribute to the available supply of cord blood and to offer state residents the option of easily donating to a public cord blood bank. The Committee further agreed that Connecticut should add cord blood collection and banking to its menu of biotechnological activities to complement existing programs such as stem cell research.

The October 30, 2006 meeting included presentations from Dr. Joanne Kurtzberg, Director of the Duke Pediatric Blood Marrow Transplant Program and the Carolinas Cord Blood Bank, Larry Smith of the Rhode Island Blood Center, Morey Kraus, Chief Technology Officer at ViaCell, Dr. Gad Lavy, Medical Director at Lifeline Cryogenics, the only private cord blood bank in Connecticut, and Dr. Dennis Todd of the Elie Katz Blood Center in New Jersey. The speakers provided the Committee with information on a variety of current public and private blood banking models.

VIII. COMMITTEE FINDINGS

At its December 5, 2006 meeting, the Committee first considered and adopted broad guidelines that should be considered in addressing the role of the State of Connecticut in public cord blood banking. The following general conclusions and recommendations regarding public cord blood banking are submitted as those guidelines:

- Connecticut should develop viable public cord blood banking options for birthing mothers.
- All birthing mothers should be informed about available public and private cord blood donation and banking options in the state.
- Connecticut efforts should address the need for racial and ethnic diversity in and unrestricted access to public umbilical cord blood supplies.
- Connecticut does not necessarily need to develop its own public cord blood donation and banking program, and should explore partnerships with existing public and/or private cord blood banks.
- The collection, transportation, processing and storage of all umbilical cord blood from birthing mothers in Connecticut should be done in accordance with all applicable existing regulatory requirements promulgated by the federal government and by national accreditation bodies.
- Relevant components of the Connecticut health care delivery community should be fully educated about public and private cord blood banking programs and as appropriate, fully compliant with acceptable standards of practice for the collection of umbilical cord blood.

The Committee next considered a range of models for state involvement, consistent with the legislative directive. Each model was examined against an analytical frame work that considered six elements, ownership, control, capital investment, operating expense risk, liability risk and investment return. The models considered and their relative merits are summarized as follows:

- A. Establish a public cord blood bank - The state would own and operate a public cord blood bank that would physically locate within the state and collect CBUs at hospitals within the state
 - a. Overview
 - i. Ownership – yes
 - ii. Control – yes
 - iii. Capital investment – yes
 - iv. Operating expense (business) risk – yes

- v. Liability – yes
 - vi. Investment return – highest opportunity
 - b. Advantages
 - i. Have ownership and control over banking operations
 - ii. Benefit from business of public banking
 - iii. Control over collection decisions
 - 1. hospitals
 - 2. volume
 - iv. Provide access to CBUs to support research
 - c. Disadvantages
 - i. Requires significant capital investment
 - ii. Would take time to establish
 - 1. Space acquisition
 - 2. Equipment purchasing and setup
 - 3. Lab procedures and training
 - 4. Collection procedures and training
 - iii. Requires ongoing operating expense funding
 - iv. Competes with existing non-government public CBBs
 - v. Political sensitivity re collection site decisions
 - vi. Political sensitivity re limitation of volume of collections
- B. Pursue a multi-state public cord blood bank collaboration – The state would enter into an agreement with one or more neighboring states to cooperatively own and operate a public cord blood bank that would collect CBUs at hospitals within the participating states
- a. Overview
 - i. Ownership – shared
 - ii. Control – shared
 - iii. Capital investment – shared
 - iv. Operating expense risk – shared
 - v. Liability – yes
 - vi. Investment opportunity - yes
 - b. Advantages
 - i. Provides access to donation within the state
 - ii. Benefit from business of public banking
 - iii. Shared capital investment
 - iv. Some control over collection decisions
 - 1. hospitals
 - 2. volume
 - v. provide access to CBUs to support research
 - c. Disadvantages
 - i. Must negotiate with one or more states which may have different levels of commitment, competing interests, and different approval processes
 - ii. Requires some capital investment
 - iii. Would take time to establish
 - 1. Negotiate compact
 - 2. Space acquisition
 - 3. Equipment purchasing and setup
 - 4. Lab procedures and training
 - 5. Collection procedures and training
 - iv. Requires ongoing operating expense funding
 - v. Competes with existing non-government public CBBs
 - vi. Shared decision making
 - vii. Political sensitivity re collection site decisions
 - viii. Political sensitivity re limitation of volume of collections

- C. Pursue a public/private partnership for public banking – The state would enter into an agreement with an existing CBB that is engaged in public banking to establish collection operations within the state
- a. Model 1 – State ownership – the state would contract with a CBB to collect, process, store and sell CBUs on the state’s behalf. The state would retain ownership to the CBUs.
- i. Overview
 1. Ownership of CBUs – yes
 2. Control of CBUs – yes
 3. Capital investment – no
 4. Operating expense risk – yes
 5. Liability – shared?
 6. Investment return – opportunity with least investment
 - ii. Advantages
 1. Have ownership and control over CBUs
 2. Avoid capital investment
 3. Less time to establish
 4. Benefit from business of public banking
 5. Control over collection decisions
 - a. hospitals
 - b. volume
 6. provide access to CBUs to support research
 - iii. Disadvantages
 1. Requires ongoing operating expense funding
 2. Political sensitivity re collection site decisions
 3. Political sensitivity re limitation of volume of collections
- b. Model 2 – Shared ownership – the state would enter into a partnership with a CBB to co-own CBUs collected within the state
- i. Overview
 1. Ownership of CBUs – shared
 2. Control of CBUs – shared
 3. Capital investment – no
 4. Operating expense risk – shared?
 5. Liability – shared?
 6. Investment return – shared
 - ii. Advantages
 1. Have shared ownership and control over CBUs
 2. Avoid capital investment
 3. Less time to establish
 4. Benefit from business of public banking
 5. Shared control over collection decisions
 - a. hospitals
 - b. volume
 6. provide access to CBUs to support research
 - iii. Disadvantages
 1. Requires ongoing operating expense funding
 2. Shared decision making on collection decisions
 3. Political sensitivity re collection site decisions
 4. Political sensitivity re limitation of volume of collections
- c. Model 3 – Private ownership – state would contract with a CBB to establish collection services within the state;
- i. Overview

1. Ownership of CBUs – no
 2. Control of CBUs – no
 3. Capital investment – no
 4. Operating expense risk – possible
 5. Liability – no
 6. Investment return – none
- ii. Advantages
1. Establish collection within state
 2. Avoid capital investment
 3. Less time to establish
 4. Some control over collection decisions
 - a. hospitals
 - b. volume
 5. provide access to CBUs to support research
- iii. Disadvantages
1. May require ongoing operating expense funding
 2. Limited opportunity to benefit from business
 3. Political sensitivity re collection site decisions
 4. Political sensitivity re limitation of volume of collections
- d. Model 4 – Model 3 Hybrid – state contracts with CBB to establish collection services within the state; CBB would own the CBU; state may underwrite some or all of the costs of collection; state would be paid share of each CBU sold
- i. Same analysis as Model 3 except that state would benefit from some offset to investment over time

IX. COMMITTEE RECOMMENDATIONS

It is the consensus of the Committee that the most advantageous approach to providing for collection of CBUs within the state is through a public-private partnership between Connecticut and a public cord blood bank that is willing to establish public collection operations within the state to collect CBUs for therapeutic and research use. The chief advantages of this approach are that it provides a mechanism that will encourage collections within Connecticut at the earliest possible time frame while avoiding the need to invest significantly in the development of the infrastructure to create a new banking facility.

As described above, there are a number of approaches to a public-private partnership. The Committee believes that the best model would be one that provides for financial support from the state to encourage collection activity coupled with an expectation of a return to the state of some or all of the sale proceeds from CBUs collected within the state. Because the Committee was not tasked with seeking proposals for establishing collection activity, the specific terms of such an arrangement have not been determined. However, within the public-private partnership approach, the Committee recommends that the state pursue a solicitation process to seek proposals from existing banks that would contain the following elements:

1. The bank would agree to establish and operate one or more collections sites within the state to collect a targeted number of units to be determined as a part of the process.
2. The collection program would be committed to collection of CBUs that reflect the racial and ethnic and diversity of the state's citizens.
3. The bank may request an investment from the state to support collection, processing and banking.
4. Any requested state support would be offset by a proportional distribution to the state from sales of the CBUs from the partner bank, either out of the bank's general inventory or from the CBUs collected under that state program.

5. The bank would agree to set up operations within six months of the completion of the contract, provided that contracts with collection sites can be identified and negotiated within that time frame.
6. The bank would participate in the CBCC by listing units so that the maximum opportunity for use will be assured.
7. The bank must have a program that provides CBUs for research and will agree to provide units not suitable for therapeutic use to researchers located within Connecticut without charge.
8. The bank must be accredited as a public bank by one of the national accrediting organizations recognized by HRSA.
9. The bank must demonstrate ability to meet applicable FDA requirements.

It is anticipated that CBBs will be willing to establish collection sites within Connecticut under these terms. The advantages to the state in such an arrangement are:

1. Avoid the cost of establishing its own facilities.
2. Move quickly to establish collections within the state by taking advantage of the existing programs of public cord blood banks.
3. Recognizing that some investment may be necessary to encourage collection within the state, the program would hope to recoup some or all of the investment overtime by receiving a portion of the fees charged within units are sold for therapeutic or research purposes.
4. Through its participation, the state will be able to provide for collection in an equitable manner for its residents.

The committee considered the option whereby the state would establish a banking operation under state ownership and control but felt that this option was not desirable as it represented the highest cost and greatest risk to the state. In addition, it would take a considerable amount of time to set up. Since nongovernmental entities have already created expertise and capacity for public banking, the state could avoid the cost and risk but still accommodate the desire for public banking within the state through a public-private partnership.

Likewise, the committee does not recommend pursuing a multi-state approach at this time. While this approach may have advantages in providing an opportunity to share the risk of investment and operations, it would require significant effort to enlist and develop such an arrangement with other states, even if the reception to such an idea were welcome. However, the state should be prepared to respond to overtures along these lines should interest be expressed as it pursues its own track, consistent with the recommendations of the committee, if adopted.

APPENDIX A
PUBLIC ACT 06-77

PUBLIC ACT 06-77

Public Act No. 06-77

AN ACT DESIGNATING THE MONTH OF NOVEMBER AS LUNG CANCER AWARENESS MONTH AND CONCERNING THE ESTABLISHMENT OF A PUBLIC UMBILICAL CORD BLOOD BANK.

Be it enacted by the Senate and House of Representatives in General Assembly convened:

Section 1. Subsection (a) of section 10-29a of the 2006 supplement to the general statutes is amended by adding subdivision (51) as follows (*Effective October 1, 2006*):

(NEW) (51) Lung Cancer Awareness Month. The Governor shall proclaim the month of November to be Lung Cancer Awareness Month to heighten public awareness of the fact that lung cancer is the leading cause of cancer death of both men and women in the United States. Suitable exercises shall be held in the State Capitol and elsewhere as the Governor designates for the observance of the month.

Sec. 2. (*Effective from passage*) (a) The Commissioner of Public Health, in consultation with the Stem Cell Research Advisory Committee established pursuant to section 19a-32f of the 2006 supplement to the general statutes, shall establish an ad hoc committee to examine and evaluate the feasibility of (1) establishing a public umbilical cord blood bank for the purpose of collecting and storing umbilical cord blood and placental tissue donated by maternity patients at hospitals licensed in this state, (2) entering into a multistate public umbilical cord collaboration, and (3) developing a public-private partnership with existing umbilical cord blood banks. The committee shall hold its first meeting not later than sixty days after the effective date of this section. Other topics may be included at the discretion of either the commissioner or the Stem Cell Research Advisory Committee.

(b) (1) The ad hoc committee shall be appointed by the Commissioner of Public Health and shall consist of the Commissioners of Public Health and Economic and Community Development, or their designees; one member of the Stem Cell Research Advisory Committee established pursuant to section 19a-32f of the 2006 supplement to the general statutes, selected by the Stem Cell Research Advisory Committee; one researcher from a private institution of higher education in the state; one researcher from a public institution of higher education in the state; one representative of an educational and business support network organization for bioscience in the state; one individual who is a member in good standing of the American Association of Blood Banks, with expertise in umbilical cord blood banking and the Food and Drug Administration's federal safety standards for umbilical cord blood banks; one individual with multiple years of experience in establishing, executing and administering an umbilical cord blood registry. The Commissioner of Public Health shall serve as chairperson of the committee.

(2) The Commissioner of Public Health, in consultation with the Stem Cell Research Advisory Committee, may expand the membership of the ad hoc committee to include additional members if either decides such expansion would be useful.

(c) On or before January 5, 2007, the Commissioner of Public Health shall submit, in accordance with section 11-4a of the general statutes, the results of the examination, along with any recommendations, to the Governor and the joint standing committee of the General Assembly having cognizance of matters relating to public health.

APPENDIX B
UMBILICAL CORD BLOOD BANK AD HOC
COMMITTEE MEMBERSHIP

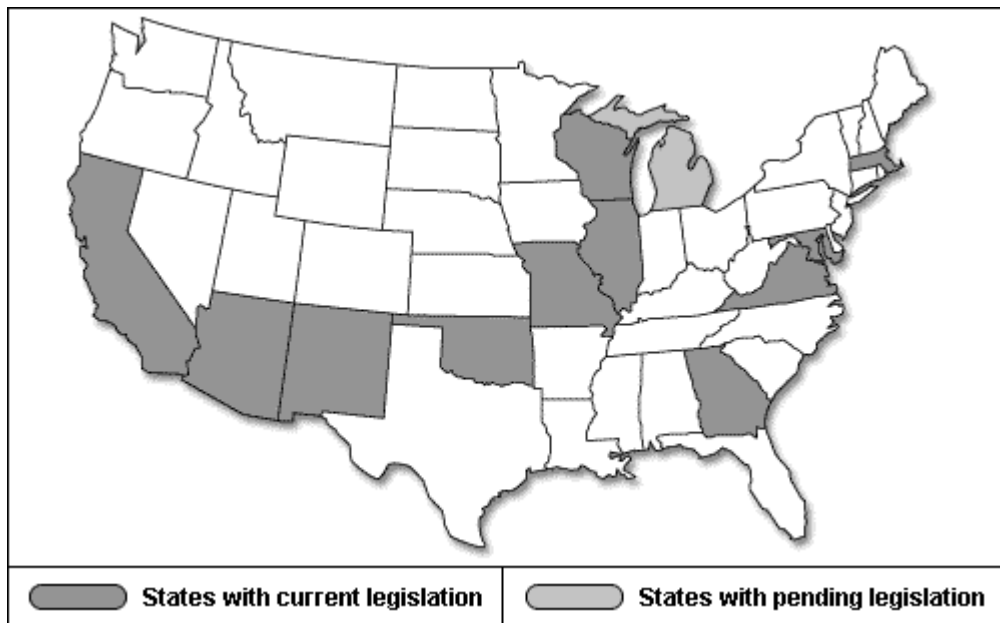
Umbilical Cord Blood Bank Ad Hoc Committee Membership

	APPOINTEE NAME/ADDRESS
Stated in the PA	<p>Commissioner J. Robert Galvin, M.D., Chair Department of Public Health (DEP) 410 Capitol Ave. Hartford, CT 06106 robert.galvin@po.state.ct.us Blanche Mink: blanche.mink@po.state.ct.us</p>
Stated in the PA	<p>Commissioner James Abromaitis Department of Economic & Community Development (DECD) 505 Hudson St. Hartford, CT 06106-7107 james.abromaitis@po.state.ct.us Edward Bona: Edward.bona@po.state.ct.us</p>
Stem Cell Research Advisory Committee Member	<p>M. William Lensch, Ph.D. Division of Hematology/Oncology Children's Hospital Boston, Karp 7004 300 Longwood Avenue Boston, MA 02115 Mathew.Lensch@childrens.harvard.edu</p>
Researcher Public Institution of Higher Education	<p>Marc Lalande, Ph.D., Professor and Chair, Dept. of Genetics and Developmental Biology University of CT Health Center 263 Farmington Ave., Rm ARB E-3032, Farmington, CT 06030 lalande@uch.edu</p>
Years of Experience with Umbilical Cord Blood Registry	<p>Gad Lavy, M.D., F.A.C.O.G., Medical Director Lifeline Cryogenics 1275 Summer St., Suite 201 Stamford, CT 06905 glavy@lifelinecryogenics.com</p>
Educational and Business Support Network Organization for Bioscience	<p>Paul Pescatello, J.D., Ph.D. CEO/President CT United for Research Excellence 300 George St., Suite 561 New Haven, CT 06511 ppescatello@curenet.org</p>
AABB Member in Good Standing, Expertise in UCBB and FDA Standards	<p>Patricia Pisciotto, M.D., Medical Director American Red Cross 209 Farmington Ave. Farmington, CT 06032 pisciottopt@usa.redcross.org</p>
Researcher Private Institution of Higher Education	<p>Edward Snyder, M.D., F.A.C.P. Yale-New Haven Hospital Blood Bank, CB459, 20 York St. New Haven, CT 06504 edward.snyder@yale.edu</p>
Additional Expert	<p>Michael Boo, Strategic Development Officer National Marrow Donor Program 3001 Broadway St., NE Minneapolis, MN 55413 mboo@nmdp.org</p>

APPENDIX C
OTHER STATE REGULATORY ACTIVITIES

Cord Blood Legislation: State by State

Each year more than 35,000 American children and adults with life threatening illnesses find themselves in need of a stem cell transplant. The growing need for a suitable stem cell match has garnered the attention of state lawmakers. In response to their constituents, state legislators across the country are introducing legislation intended to help physicians and expectant parents on the options for donating or banking lifesaving newborn stem cells.



States with current cord blood legislation

Arizona

Beginning in January 2007, health care professionals will be required to inform a pregnant patient about her ability to family bank or donate her newborn's cord blood. The Umbilical Cord Blood; Donation; Information Act will advise expectant mothers about the benefits of umbilical cord blood collection to the newborn and immediate biological family. Arizona is the first state to inform expectant parents about free cord blood collection and storage programs offered by family and sibling donor banks.

California

Consistent with the recommendations of the Institute of Medicine (IOM) the Maternal and Child Health Advancement Act will authorize the

States with pending cord blood legislation

Michigan

There are several cord blood bills pending in Michigan. The first would establish a state wide network of qualified cord blood banks. The second bill is an education initiative on all cord blood banking options for pregnant women and the general public.

Department of Health to create a cord blood awareness campaign that will offer standardized, objective information to expectant mothers about the differences between public and private banking, current and future uses of cord blood, and how medical or family history can impact a family's decision to donate or family bank their newborn's stem cells.

Georgia

The Governor issued an executive order to establish the Delivering the Cure: Newborn Umbilical Cord Blood Initiative Act to establish a commission whose task will include promoting awareness and encouraging donation of postnatal tissue and fluid to public or private difference between public and private banking programs; the medical process involved in the collection and storage of postnatal tissue and fluid; the current and potential future medical uses of stored postnatal tissue and fluid; the benefits and risks involved in the banking of postnatal tissue and fluid; and the availability and cost of storing postnatal tissue and fluid in public and private umbilical cord blood banks.

Illinois

In 2004, the Hospital Licensing Act was amended to add a mandate that hospitals offer pregnant women the option to donate their newborn's cord blood to a public bank.

Maryland

The Maryland Department of Health and Mental Hygiene - Umbilical Cord Blood Donation - Educational Materials Act would require the Department of Health to develop educational materials about cord blood donation and would require specified obstetricians and hospitals to distribute specified educational materials to specified patients; etc.

Massachusetts

An Act Enhancing Regenerative Medicine in the MA Commonwealth has become law and has been incorporated into the Acts that govern the state. The Massachusetts Department of Public Health will establish a program to educate women on public and private cord blood banking options and their differences. Hospitals within the commonwealth will inform pregnant patients of their ability to donate to a public bank.

Missouri

Currently unfunded the Establishes the Criteria for Grants to Umbilical Cord Blood Banks Act will expand existing cord blood banks and establish new ones in the sate of Missouri.

New Mexico

Umbilical Cord Blood Banking Act requires

physicians to inform patients about cord blood donation and requires the Department of Health to prepare and distribute written publications informing expectant mothers about cord blood donation.

Oklahoma

The Danielle Martinez Act, named for a 7-year-old leukemia patient establishes an advisory council to develop recommendations for a cord blood donor program.

Virginia

The Virginia Cord Blood Initiative Act establishes the Virginia Cord Blood Bank Initiative as a public resource for treating patients with life threatening illnesses and for use in advancing basic and clinical research. Women will be offered the opportunity to donate cord blood. The initiative will do research and outreach particularly for ethnic and racial minorities. Information will be disseminated through health departments and Medicaid.

Wisconsin

The Donation of Umbilical Cord Blood Act requires the principal prenatal healthcare provider of a pregnant woman to offer her information prior to the 35th week of pregnancy about her option to donate umbilical cord blood.

APPENDIX D
STEM CELL THERAPEUTIC AND RESEARCH ACT OF 2005

Stem Cell Therapeutic and Research Act of 2005

PUBLIC LAW 109–129—DEC. 20, 2005

119 STAT. 2550 PUBLIC LAW 109–129—DEC. 20, 2005

Public Law 109–129

109th Congress

An Act

To provide for the collection and maintenance of human cord blood stem cells for the treatment of patients and research, and to amend the Public Health Service Act to authorize the C.W. Bill Young Cell Transplantation Program.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the “Stem Cell Therapeutic and Research Act of 2005”.

SEC. 2. CORD BLOOD INVENTORY.

(a) IN GENERAL.—The Secretary of Health and Human Services shall enter into one-time contracts with qualified cord blood banks to assist in the collection and maintenance of 150,000 new units of high-quality cord blood to be made available for transplantation through the C.W. Bill Young Cell Transplantation Program and to carry out the requirements of subsection (b).

(b) REQUIREMENTS.—The Secretary shall require each recipient of a contract under this section—

- (1) to acquire, tissue-type, test, cryopreserve, and store donated units of cord blood acquired with the informed consent of the donor, as determined by the Secretary pursuant to section 379(c) of the Public Health Service Act, in a manner that complies with applicable Federal and State regulations;
- (2) to encourage donation from a genetically diverse population;
- (3) to make cord blood units that are collected pursuant to this section or otherwise and meet all applicable Federal standards available to transplant centers for transplantation;
- (4) to make cord blood units that are collected, but not appropriate for clinical use, available for peer-reviewed research;
- (5) to make data available, as required by the Secretary and consistent with section 379(d)(3) of the Public Health Service Act (42 U.S.C. 274k(d)(3)), as amended by this Act, in a standardized electronic format, as determined by the Secretary, for the C.W. Bill Young Cell Transplantation Program; and
- (6) to submit data in a standardized electronic format for inclusion in the stem cell therapeutic outcomes database maintained under section 379A of the Public Health Service Act, as amended by this Act.

(c) RELATED CORD BLOOD DONORS.—

(1) IN GENERAL.—The Secretary shall establish a 3-year demonstration project under which qualified cord blood banks receiving a contract under this section may use a portion of the funding under such contract for the collection and storage of cord blood units for a family where a first-degree relative has been diagnosed with a condition that will benefit from transplantation (including selected blood disorders, malignancies, metabolic storage disorders, hemoglobinopathies, and congenital immunodeficiencies) at no cost to such family. Qualified cord blood banks collecting cord blood units under this paragraph shall comply with the requirements of paragraphs (1), (2), (3), and (5) of subsection (b).

(2) AVAILABILITY.—Qualified cord blood banks that are operating a program under paragraph (1) shall provide assurances that the cord blood units in such banks will be available for directed transplantation until such time that the cord blood unit is released for transplantation or is transferred by the family to the C.W. Bill Young Cell Transplantation Program in accordance with guidance or regulations promulgated by the Secretary.

(3) INVENTORY.—Cord blood units collected through the program under this section shall not be counted toward the 150,000 inventory goal under the C.W. Bill Young Cell Transplantation Program.

(4) REPORT.—Not later than 90 days after the date on which the project under paragraph (1) is terminated by the Secretary, the Secretary shall submit to Congress a report on the outcomes of the project that shall include the recommendations of the Secretary with respect to the continuation of such project.

(d) APPLICATION.—To seek to enter into a contract under this section, a qualified cord blood bank shall submit an application to the Secretary at such time, in such manner, and containing such information as the Secretary may reasonably require. At a minimum, an application for a contract under this section shall include a requirement that the applicant—

(1) will participate in the C.W. Bill Young Cell Transplantation Program for a period of at least 10 years;

(2) will make cord blood units collected pursuant to this section available through the C.W. Bill Young Cell Transplantation Program in perpetuity or for such time as determined viable by the Secretary; and

(3) if the Secretary determines through an assessment, or through petition by the applicant, that a cord blood bank is no longer operational or does not meet the requirements of section 379(d)(4) of the Public Health Service Act (as added by this Act) and as a result may not distribute the units, transfer the units collected pursuant to this section to another qualified cord blood bank approved by the Secretary to ensure continued availability of cord blood units.

(e) DURATION OF CONTRACTS.—

(1) IN GENERAL.—Except as provided in paragraph (2), the term of each contract entered into by the Secretary under this section shall be for 10 years. The Secretary shall ensure that no Federal funds shall be obligated under any such contract after the earlier of—

(A) the date that is 3 years after the date on which the contract is entered into; or

(B) September 30, 2010.

(2) EXTENSIONS.—Subject to paragraph (1)(B), the Secretary may extend the period of funding under a contract under this section to exceed a period of 3 years if—

(A) the Secretary finds that 150,000 new units of high quality cord blood have not yet been collected pursuant to this section; and

(B) the Secretary does not receive an application for a contract under this section from any qualified cord blood bank that has not previously entered into a contract under this section or the Secretary determines that the outstanding inventory need cannot be met by the one or more qualified cord blood banks that have submitted an application for a contract under this section.

(3) PREFERENCE.—In considering contract extensions under paragraph (2), the Secretary shall give preference to qualified cord blood banks that the Secretary determines have demonstrated a superior ability to satisfy the requirements described in subsection (b) and to achieve the overall goals for which the contract was awarded.

(f) REGULATIONS.—The Secretary may promulgate regulations to carry out this section.

(g) DEFINITIONS.—In this section:

(1) The term “C.W. Bill Young Cell Transplantation Program” means the C.W. Bill Young Cell Transplantation Program under section 379 of the Public Health Service Act, as amended by this Act.

(2) The term “cord blood donor” means a mother who has delivered a baby and consents to donate the neonatal blood remaining in the placenta and umbilical cord after separation from the newborn baby.

(3) The term “cord blood unit” means the neonatal blood collected from the placenta and umbilical cord of a single newborn baby.

(4) The term “first-degree relative” means a sibling or parent who is one meiosis away from a particular individual in a family.

(5) The term “qualified cord blood bank” has the meaning given to that term in section 379(d)(4) of the Public Health Service Act, as amended by this Act.

(6) The term “Secretary” means the Secretary of Health and Human Services.

(h) AUTHORIZATION OF APPROPRIATIONS.—

(1) EXISTING FUNDS.—Any amounts appropriated to the Secretary for fiscal year 2004 or 2005 for the purpose of assisting in the collection or maintenance of cord blood shall remain available to the Secretary until the end of fiscal year 2007.

(2) **SUBSEQUENT FISCAL YEARS.**—There are authorized to be appropriated to the Secretary \$15,000,000 for each of fiscal years 2007, 2008, 2009, and 2010 to carry out this section.

(3) **LIMITATION.**—Not to exceed 5 percent of the amount appropriated under this section in each of fiscal years 2007 through 2009 may be used to carry out the demonstration project under subsection (c).

SEC. 3. C.W. BILL YOUNG CELL TRANSPLANTATION PROGRAM.

(a) **NATIONAL PROGRAM.**—Section 379 of the Public Health Service Act (42 U.S.C. 274k) is amended to read as follows:

“SEC. 379. NATIONAL PROGRAM.

“(a) **ESTABLISHMENT.**—The Secretary, acting through the Administrator of the Health Resources and Services Administration, shall by one or more contracts establish and maintain a C.W. Bill Young Cell Transplantation Program (referred to in this section as the ‘Program’), successor to the National Bone Marrow Donor Registry, that has the purpose of increasing the number of transplants for recipients suitably matched to biologically unrelated donors of bone marrow and cord blood, and that meets the requirements of this section. The Secretary may award a separate contract to perform each of the major functions of the Program described in paragraphs (1) and (2) of subsection (d) if deemed necessary by the Secretary to operate an effective and efficient system that is in the best interest of patients. The Secretary shall conduct a separate competition for the initial establishment of the cord blood functions of the Program. The Program shall be under the general supervision of the Secretary. The Secretary shall establish an Advisory Council to advise, assist, consult with, and make recommendations to the Secretary on matters related to the activities carried out by the Program. The members of the Advisory Council shall be appointed in accordance with the following:

“(1) Each member of the Advisory Council shall serve for a term of 2 years, and each such member may serve as many as 3 consecutive 2-year terms, except that—

“(A) such limitations shall not apply to the Chair of the Advisory Council (or the Chair-elect) or to the member of the Advisory Council who most recently served as the Chair; and “(B) one additional consecutive 2-year term may be served by any member of the Advisory Council who has no employment, governance, or financial affiliation with any donor center, recruitment organization, transplant center, or cord blood bank.

“(2) A member of the Advisory Council may continue to serve after the expiration of the term of such member until a successor is appointed.

“(3) In order to ensure the continuity of the Advisory Council, the Advisory Council shall be appointed so that each year the terms of approximately one-third of the members of the Advisory Council expire.

“(4) The membership of the Advisory Council—

“(A) shall include as voting members a balanced number of representatives including representatives of marrow donor centers and marrow transplant centers, representatives of cord blood banks and participating birthing hospitals, recipients of a bone marrow transplant, recipients of a cord blood transplant, persons who require such transplants, family members of such a recipient or family members of a patient who has requested the assistance of the Program in searching for an unrelated donor of bone marrow or cord blood, persons with expertise in bone marrow and cord blood transplantation, persons with expertise in typing, matching, and transplant outcome data analysis, persons with expertise in the social sciences, basic scientists with expertise in the biology of adult stem cells, and members of the general public; and

“(B) shall include as nonvoting members representatives from the Department of Defense Marrow Donor Recruitment and Research Program operated by the Department of the Navy, the Division of Transplantation of the Health Resources and Services Administration, the Food and Drug Administration, and the National Institutes of Health.

“(5) Members of the Advisory Council shall be chosen so as to ensure objectivity and balance and reduce the potential for conflicts of interest. The Secretary shall establish bylaws and procedures—

“(A) to prohibit any member of the Advisory Council who has an employment, governance, or financial affiliation with a donor center, recruitment organization, transplant center, or cord blood bank from participating in any decision that materially affects the center, recruitment organization, transplant center, or cord blood bank; and

“(B) to limit the number of members of the Advisory Council with any such affiliation.

“(6) The Secretary, acting through the Advisory Council, shall submit to the Congress—

“(A) an annual report on the activities carried out under this section; and

“(B) not later than 6 months after the date of the enactment of the Stem Cell Therapeutic and Research Act of 2005, a report of recommendations on the scientific factors necessary to define a cord blood unit as a high-quality unit.

“(b) ACCREDITATION.—The Secretary shall, through a public process, recognize one or more accreditation entities for the accreditation of cord blood banks.

“(c) INFORMED CONSENT.—The Secretary shall, through a public process, examine issues of informed consent, including—

“(1) the appropriate timing of such consent; and

“(2) the information provided to the maternal donor regarding all of her medically appropriate cord blood options. Based on such examination, the Secretary shall require that the standards used by the accreditation entities recognized under subsection (b) ensure that a cord blood unit is acquired with the informed consent of the maternal donor.

“(d) FUNCTIONS.—

“(1) BONE MARROW FUNCTIONS.—With respect to bone marrow, the Program shall—

“(A) operate a system for identifying, matching, and facilitating the distribution of bone marrow that is suitably matched to candidate patients;

“(B) consistent with paragraph (3), permit transplant physicians, other appropriate health care professionals, and patients to search by means of electronic access all available bone marrow donors listed in the Program;

“(C) carry out a program for the recruitment of bone marrow donors in accordance with subsection (e), including with respect to increasing the representation of racial and ethnic minority groups (including persons of mixed ancestry) in the enrollment of the Program;

“(D) maintain and expand medical contingency response capabilities, in coordination with Federal programs, to prepare for and respond effectively to biological, chemical, or radiological attacks, and other public health emergencies that can damage marrow, so that the capability of supporting patients with marrow damage from disease can be used to support casualties with marrow damage;

“(E) carry out informational and educational activities in accordance with subsection (e);

“(F) at least annually update information to account for changes in the status of individuals as potential donors of bone marrow;

“(G) provide for a system of patient advocacy through the office established under subsection (h);

“(H) provide case management services for any potential donor of bone marrow to whom the Program has provided a notice that the potential donor may be suitably matched to a particular patient through the office established under subsection (h);

“(I) with respect to searches for unrelated donors of bone marrow that are conducted through the system under subparagraph (A), collect, analyze, and publish data in a standardized electronic format on the number and percentage of patients at each of the various stages of the search process, including data regarding the furthest stage reached, the number and percentage of patients who are unable to complete the search process, and the reasons underlying such circumstances;

“(J) support studies and demonstration and outreach projects for the purpose of increasing the number of individuals who are willing to be marrow donors to ensure a genetically diverse donor pool; and

“(K) facilitate research with the appropriate Federal agencies to improve the availability, efficiency, safety, and cost of transplants from unrelated donors and the effectiveness of Program operations.

“(2) CORD BLOOD FUNCTIONS.—With respect to cord blood, the Program shall—

“(A) operate a system for identifying, matching, and facilitating the distribution of donated cord blood units that are suitably matched to candidate patients and meet all applicable Federal and State regulations (including informed consent and Food and Drug Administration regulations) from a qualified cord blood bank;

“(B) consistent with paragraph (3), allow transplant physicians, other appropriate health care professionals, and patients to search by means of electronic access all available cord blood units made available through the Program;

“(C) allow transplant physicians and other appropriate health care professionals to reserve, as defined by the Secretary, a cord blood unit for transplantation;

“(D) support studies and demonstration and outreach projects for the purpose of increasing cord blood donation to ensure a genetically diverse collection of cord blood units;

“(E) provide for a system of patient advocacy through the office established under subsection (h);

“(F) coordinate with the qualified cord blood banks to support informational and educational activities in accordance with subsection (g);

“(G) maintain and expand medical contingency response capabilities, in coordination with Federal programs, to prepare for and respond effectively to biological, chemical, or radiological attacks, and other public health emergencies that can damage marrow, so that the capability of supporting patients with marrow damage from disease can be used to support casualties with marrow damage; and

“(H) with respect to the system under subparagraph (A), collect, analyze, and publish data in a standardized electronic format, as required by the Secretary, on the number and percentage of patients at each of the various stages of the search process, including data regarding the furthest stage reached, the number and percentage of patients who are unable to complete the search process, and the reasons underlying such circumstances.

“(3) SINGLE POINT OF ACCESS; STANDARD DATA.—

“(A) SINGLE POINT OF ACCESS.—The Secretary shall ensure that health care professionals and patients are able to search electronically for and facilitate access to, in the manner and to the extent defined by the Secretary and consistent with the functions described in paragraphs (1)(A) and (2)(A), cells from bone marrow donors and cord blood units through a single point of access.

“(B) STANDARD DATA.—The Secretary shall require all recipients of contracts under this section to make available a standard dataset for purposes of subparagraph (A) in a standardized electronic format that enables transplant physicians to compare among and between bone marrow donors and cord blood units to ensure the best possible match for the patient.

“(4) DEFINITION.—The term ‘qualified cord blood bank’

means a cord blood bank that—

“(A) has obtained all applicable Federal and State licenses, certifications, registrations (including pursuant to the regulations of the Food and Drug Administration), and other authorizations required to operate and maintain a cord blood bank;

“(B) has implemented donor screening, cord blood collection practices, and processing methods intended to protect the health and safety of donors and transplant recipients to improve transplant outcomes, including with respect to the transmission of potentially harmful infections and other diseases;

“(C) is accredited by an accreditation entity recognized by the Secretary under subsection (b);

“(D) has established a system of strict confidentiality to protect the identity and privacy of patients and donors in accordance with existing Federal and State law;

“(E) has established a system for encouraging donation by a genetically diverse group of donors; and

“(F) has established a system to confidentially maintain linkage between a cord blood unit and a maternal donor.

“(e) BONE MARROW RECRUITMENT; PRIORITIES; INFORMATION AND EDUCATION.—

“(1) RECRUITMENT; PRIORITIES.—The Program shall carry out activities for the recruitment of bone marrow donors. Such recruitment program shall identify populations that are underrepresented among potential donors enrolled with the Program. In the case of populations that are identified under the preceding sentence:

“(A) The Program shall give priority to carrying out activities under this part to increase representation for such populations in order to enable a member of such a population, to the extent practicable, to have a probability of finding a suitable unrelated donor that is comparable to the probability that an individual who is not a member of an underrepresented population would have.

“(B) The Program shall consider racial and ethnic minority groups (including persons of mixed ancestry) to be populations that have been identified for purposes of this paragraph, and shall carry out subparagraph (A) with respect to such populations.

“(2) INFORMATION AND EDUCATION REGARDING RECRUITMENT; TESTING AND ENROLLMENT.—

“(A) IN GENERAL.—The Program shall carry out informational and educational activities, in coordination with organ donation public awareness campaigns operated through the Department of Health and Human Services, for purposes of recruiting individuals to serve as donors of bone marrow, and shall test and enroll with the Program potential bone marrow donors. Such information and educational activities shall include the following:

“(i) Making information available to the general public, including information describing the needs of patients with respect to donors of bone marrow.

“(ii) Educating and providing information to individuals who are willing to serve as potential bone marrow donors.

“(iii) Training individuals in requesting individuals to serve as potential bone marrow donors.

“(B) PRIORITIES.—In carrying out informational and educational activities under subparagraph (A), the Program shall give priority to recruiting individuals to serve as donors of bone marrow for populations that are identified under paragraph (1).

“(3) TRANSPLANTATION AS TREATMENT OPTION.—In addition to activities regarding recruitment, the recruitment program under paragraph (1) shall provide information to physicians, other health care professionals, and the public regarding bone marrow transplants from unrelated donors as a treatment option.

“(4) IMPLEMENTATION OF SUBSECTION.—The requirements of this subsection shall be carried out by the entity that has been awarded a contract by the Secretary under subsection (a) to carry out the functions described in subsection (d)(1).

“(f) BONE MARROW CRITERIA, STANDARDS, AND PROCEDURES.—

The Secretary shall enforce, for participating entities, including the Program, individual marrow donor centers, marrow donor registries, marrow collection centers, and marrow transplant centers—

“(1) quality standards and standards for tissue typing, obtaining the informed consent of donors, and providing patient advocacy;

“(2) donor selection criteria, based on established medical criteria, to protect both the donor and the recipient and to prevent the transmission of potentially harmful infectious diseases such as the viruses that cause hepatitis and the etiologic agent for Acquired Immune Deficiency Syndrome;

“(3) procedures to ensure the proper collection and transportation of the marrow;

“(4) standards for the system for patient advocacy operated under subsection (h), including standards requiring the provision of appropriate information (at the start of the search process and throughout the process) to patients and their families and physicians;

“(5) standards that—

“(A) require the establishment of a system of strict confidentiality of records relating to the identity, address, HLA type, and managing marrow donor center for marrow donors and potential marrow donors; and

“(B) prescribe the purposes for which the records described in subparagraph (A) may be disclosed, and the circumstances and extent of the disclosure; and

“(6) in the case of a marrow donor center or marrow donor registry participating in the program, procedures to ensure the establishment of a method for integrating donor files, searches, and general procedures of the center or registry with the Program.

“(g) CORD BLOOD RECRUITMENT; PRIORITIES; INFORMATION AND EDUCATION.—

“(1) RECRUITMENT; PRIORITIES.—The Program shall support activities, in cooperation with qualified cord blood banks, for the recruitment of cord blood donors. Such recruitment program shall identify populations that are underrepresented among cord blood donors. In the case of populations that are identified under the preceding sentence:

“(A) The Program shall give priority to supporting activities under this part to increase representation for such populations in order to enable a member of such a population, to the extent practicable, to have a probability of finding a suitable cord blood unit that is comparable to the probability that an individual who is not a member of an underrepresented population would have.

“(B) The Program shall consider racial and ethnic minority groups (including persons of mixed ancestry) to be populations that have been identified for purposes of this paragraph, and shall support activities under subparagraph (A) with respect to such populations.

“(2) INFORMATION AND EDUCATION REGARDING RECRUITMENT; TESTING AND DONATION.—

“(A) IN GENERAL.—In carrying out the recruitment program under paragraph (1), the Program shall support informational and educational activities in coordination with qualified cord blood banks and organ donation public awareness campaigns operated through the Department of Health and Human Services, for purposes of recruiting pregnant women to serve as donors of cord blood. Such information and educational activities shall include the following:

“(i) Making information available to the general public, including information describing the needs of patients with respect to cord blood units.

“(ii) Educating and providing information to pregnant women who are willing to donate cord blood units.

“(iii) Training individuals in requesting pregnant women to serve as cord blood donors.

“(B) PRIORITIES.—In carrying out informational and educational activities under subparagraph (A), the Program shall give priority to supporting the recruitment of pregnant women to serve as donors of cord blood for populations that are identified under paragraph (1).

“(3) TRANSPLANTATION AS TREATMENT OPTION.—In addition to activities regarding recruitment, the recruitment program under paragraph (1) shall provide information to physicians, other health care professionals, and the public regarding cord blood transplants from donors as a treatment option.

“(4) IMPLEMENTATION OF SUBSECTION.—The requirements of this subsection shall be carried out by the entity that has been awarded a contract by the Secretary under subsection (a) to carry out the functions described in subsection (d)(2).

“(h) PATIENT ADVOCACY AND CASE MANAGEMENT FOR BONE MARROW AND CORD BLOOD.—

“(1) IN GENERAL.—The Secretary shall establish and maintain, through a contract or other means determined appropriate by the Secretary, an office of patient advocacy (in this subsection referred to as the ‘Office’).

“(2) GENERAL FUNCTIONS.—The Office shall meet the following requirements:

“(A) The Office shall be headed by a director.

“(B) The Office shall be staffed by individuals with expertise in bone marrow and cord blood therapy covered under the Program.

“(C) The Office shall operate a system for patient advocacy, which shall be separate from mechanisms for donor advocacy, and which shall serve patients for whom the Program is conducting, or has been requested to conduct, a search for a bone marrow donor or cord blood unit.

“(D) In the case of such a patient, the Office shall serve as an advocate for the patient by directly providing to the patient (or family members, physicians, or other individuals acting on behalf of the patient) individualized services with respect to efficiently utilizing the system under paragraphs (1) and (2) of subsection (d) to conduct an ongoing search for a bone marrow donor or cord blood unit and assist with information regarding third party payor matters.

“(E) In carrying out subparagraph (D), the Office shall monitor the system under paragraphs (1) and (2) of subsection (d) to determine whether the search needs of the patient involved are being met, including with respect to the following:

“(i) Periodically providing to the patient (or an individual acting on behalf of the patient) information regarding bone marrow donors or cord blood units that are suitably matched to the patient, and other information regarding the progress being made in the search.

“(ii) Informing the patient (or such other individual) if the search has been interrupted or discontinued.

“(iii) Identifying and resolving problems in the search, to the extent practicable.

“(F) The Office shall ensure that the following data are made available to patients:

“(i) The resources available through the Program.

“(ii) A comparison of transplant centers regarding search and other costs that prior to transplantation are charged to patients by transplant centers.

“(iii) The post-transplant outcomes for individual transplant centers.

“(iv) Information concerning issues that patients may face after a transplant.

“(v) Such other information as the Program determines to be appropriate.

“(G) The Office shall conduct surveys of patients (or family members, physicians, or other individuals acting on behalf of patients) to determine the extent of satisfaction with the system for patient advocacy under this subsection, and to identify ways in which the system can be improved to best meet the needs of patients.

“(3) CASE MANAGEMENT.—

“(A) IN GENERAL.—In serving as an advocate for a patient under paragraph (2), the Office shall provide individualized case management services directly to the patient (or family members, physicians, or other individuals acting on behalf of the patient), including—

“(i) individualized case assessment; and

“(ii) the functions described in paragraph (2)(D) (relating to progress in the search process).

“(B) POSTSEARCH FUNCTIONS.—In addition to the case management services described in paragraph (1) for patients, the Office shall, on behalf of patients who have completed the search for a bone marrow donor or cord blood unit, provide information and education on the process of receiving a transplant, including the post-transplant process.

“(i) COMMENT PROCEDURES.—The Secretary shall establish and provide information to the public on procedures under which the Secretary shall receive and consider comments from interested persons relating to the manner in which the Program is carrying out the duties of the Program. The Secretary may promulgate regulations under this section.

“(j) CONSULTATION.—In developing policies affecting the Program, the Secretary shall consult with the Advisory Council, the Department of Defense Marrow Donor Recruitment and Research Program operated by the Department of the Navy, and the board of directors of each entity awarded a contract under this section.

“(k) CONTRACTS.—

“(1) APPLICATION.—To be eligible to enter into a contract under this section, an entity shall submit to the Secretary and obtain approval of an application at such time, in such manner, and containing such information as the Secretary shall by regulation prescribe.

“(2) CONSIDERATIONS.—In awarding contracts under this section, the Secretary shall give consideration to the continued safety of donors and patients and other factors deemed appropriate by the Secretary.

“(l) ELIGIBILITY.—Entities eligible to receive a contract under this section shall include private nonprofit entities.

“(m) RECORDS.—

“(1) RECORDKEEPING.—Each recipient of a contract or subcontract under subsection (a) shall keep such records as the Secretary shall prescribe, including records that fully disclose the amount and disposition by the recipient of the proceeds of the contract, the total cost of the undertaking in connection with which the contract was made, and the amount of the portion of the cost of the undertaking supplied by other sources, and such other records as will facilitate an effective audit.

“(2) EXAMINATION OF RECORDS.—The Secretary and the Comptroller General of the United States shall have access to any books, documents, papers, and records of the recipient of a contract or subcontract entered into under this section that are pertinent to the contract, for the purpose of conducting audits and examinations.

“(n) PENALTIES FOR DISCLOSURE.—Any person who discloses the content of any record referred to in subsection (d)(4)(D) or (f)(5)(A) without the prior written consent of the donor or potential donor with respect to whom the record is maintained, or in violation of the standards described in subsection (f)(5)(B), shall be imprisoned for not more than 2 years or fined in accordance with title 18, United States Code, or both.”.

(b) STEM CELL THERAPEUTIC OUTCOMES DATABASE.—Section 379A of the Public Health Service Act (42 U.S.C. 274l) is amended to read as follows:

“SEC. 379A. STEM CELL THERAPEUTIC OUTCOMES DATABASE.

“(a) ESTABLISHMENT.—The Secretary shall by contract establish and maintain a scientific database of information relating to patients who have been recipients of a stem cell therapeutics product (including bone marrow, cord blood, or other such product) from a donor.

“(b) INFORMATION.—The outcomes database shall include information in a standardized electronic format with respect to patients described in subsection (a), diagnosis, transplant procedures, results, long-term follow-up, and such other information as the Secretary determines to be appropriate, to conduct an ongoing evaluation of the scientific and clinical status of transplantation involving recipients of a stem cell therapeutics product from a donor.

“(c) ANNUAL REPORT ON PATIENT OUTCOMES.—The Secretary shall require the entity awarded a contract under this section to submit to the Secretary an annual report concerning patient outcomes with respect to each transplant center, based on data collected and maintained by the entity pursuant to this section.

“(d) PUBLICLY AVAILABLE DATA.—The outcomes database shall make relevant scientific information not containing individually identifiable information available to the public in the form of summaries and data sets to encourage medical research and to provide information to transplant programs, physicians, patients, entities awarded a contract under section 379 donor registries, and cord blood banks.”.

(c) DEFINITIONS.—Part I of title III of the Public Health Service Act (42 U.S.C. 274k et seq.) is amended by inserting after section 379A the following:

“SEC. 379A-1. DEFINITIONS.

“In this part:

“(1) The term ‘Advisory Council’ means the advisory council established by the Secretary under section 379(a)(1).

“(2) The term ‘bone marrow’ means the cells found in adult bone marrow and peripheral blood.

“(3) The term ‘outcomes database’ means the database established by the Secretary under section 379A.

“(4) The term ‘Program’ means the C.W. Bill Young Cell Transplantation Program established under section 379.”.

(d) AUTHORIZATION OF APPROPRIATIONS.—Section 379B of the Public Health Service Act (42 U.S.C. 274m) is amended to read as follows:

“SEC. 379B. AUTHORIZATION OF APPROPRIATIONS.

“For the purpose of carrying out this part, there are authorized to be appropriated \$34,000,000 for fiscal year 2006 and \$38,000,000 for each of fiscal years 2007 through 2010.”.

(e) CONFORMING AMENDMENTS.—Part I of title III of the Public Health Service Act (42 U.S.C. 274k et seq.) is amended in the part heading, by striking “**NATIONAL BONE MARROW DONOR REGISTRY**” and inserting “**C.W. BILL YOUNG CELL TRANSPLANTATION PROGRAM**”.

SEC. 4. REPORT ON LICENSURE OF CORD BLOOD UNITS.

Not later than 90 days after the date of enactment of this Act, the Secretary of Health and Human Services, in consultation with the Commissioner of Food and Drugs, shall submit to Congress a report concerning the progress made by the Food and Administration in developing requirements for the licensing of cord blood units.

Approved December 20, 2005.

Legislative History ---H.R. 2520 (S.1317)

Senate Reports: No. 109-129 accompanying S.1317 (Comm. on Health, Education, Labor, and Pensions).
CONGRESSIONAL RECORD, Vol. 151 (2005):

May 24, considered and passed House.

Dec. 16, considered and passed Senate, amended.

Dec. 17, House concurred in Senate amendment.

APPENDIX E
SAMPLE PRO FORMA OF CORD BLOOD OPERATION

CORD BANK SIMULATION - FINANCIAL SUMMARY

Units Banked - 2,000 per year; \$1,000 subsidy/unit, 5 yrs

Rate of Sale - 1.0% of inventory

Sale Price - \$25,000 per unit

	Year 1	Year 2	Year 3	Year 4
REVENUE				
CORD UNITS SOLD	10	30	49	68
SELLING PRICE	25,000	25,000	25,000	25,000
CORD BANK REVENUE	250,000	750,000		
1,225,000	1,700,000	2,175,000		
2,625,000	3,100,000	3,550,000		
4,000,000	4,450,000			
SUBSIDIES	1,950,000	1,950,000		
1,950,000	1,950,000	1,950,000	0	0
TOTAL REVENUE	2,200,000	2,700,000		
3,175,000	3,650,000	4,125,000		
2,625,000	3,100,000	3,550,000		
4,000,000	4,450,000			
EXPENSES				
RECRUITMENT COSTS	661,375	681,216	701,653	
722,702	744,383	766,715	789,716	
813,408	837,810	862,944		
CORDS RECRUITED	6,500	6,500	6,500	6,500
PROCESSING COSTS	1,584,375	1,631,906		
1,680,284	1,729,535	1,779,683		
1,830,757	1,882,784	1,935,793		
1,989,812	2,044,873			
CORDS PROCESSED	1,950	1,950	1,950	1,950
STORAGE COSTS	41,800	23,381	25,037	26,772
FIXED COSTS	487,133	221,278	227,802	
234,406	241,094	563,538	254,730	
261,684	268,731	275,875		
SELLING COSTS	157,675	183,366	209,244	
236,381	264,833	293,479	324,704	
356,173	389,130	423,639		
TOTAL COSTS	2,932,358	2,741,147		
2,844,020	2,949,796	3,058,581		
3,555,578	3,309,368	3,427,321		
3,548,695	3,673,614			
COST PER UNIT SOLD	293,236	91,372	58,041	43,379
NET INCOME				
MARGIN (excluding subsidies)	(2,682,358)	(1,991,147)		
(1,619,020)	(1,249,796)	(883,581)		
(930,578)	(209,368)	122,679	451,305	
776,386				
CUMULATIVE MARGIN (excluding subsidies)	(2,682,358)	(4,673,505)		
(6,292,525)	(7,542,320)	(8,425,902)		
(9,356,480)	(9,565,847)	(9,443,169)		
(8,991,864)	(8,215,478)			

MARGIN (including subsidies)	(732,358)	(41,147)	330,980
700,204	1,066,419	(930,578)	
(209,368)	122,679	451,305	776,386
CUMULATIVE MARGIN (including subsidies)	(732,358)	(773,505)	
(442,525)	257,680	1,324,098	393,520
184,153	306,831	758,136	
1,534,522			