

***The Purpose of H. R. 2852 and S. 1717  
“Cord Blood Stem Cell Act of 2003”  
New York Blood Center’s Perspective***

Cord blood now promises to be one of this century’s medical miracles. Reports from many parts of the world demonstrate the rapidly growing utility of unrelated cord blood transplants for both children and adults. What used to be discarded at birth can now be collected, processed, stored and used as a source for therapeutic stem cells for a variety of devastating diseases. Unlike bone marrow, cord blood is a product on the shelf that is rapidly available for patient use. New collection and processing techniques as well as new treatment tactics such as multiple units in adults make clear that cord blood may eclipse bone marrow transplantation for hematopoietic rescue within a decade or less. The Japan Cord Blood Network has grown from 4% of total transplants when it began in 1999 to 43% in four years. Furthermore, because of its rich mix of multipotential stem cells, cord blood has an expanding range of therapeutic potential including hepatic, cardiac, neuronal and other organ system diseases.

Despite the promise of cord blood, availability for patient care has been limited because of the lack of investment resources to build inventories. Every year thousands of patients succumb to blood and immune system malignancies and genetic diseases because a bone marrow match is not found. Most of these patients might have been treatable with cord blood had the nation’s inventory been significantly larger. Moreover, the threat of terrorism and nuclear catastrophe further heightens our need for instant sources of stem cells to treat potential victims.

New York Blood Center, an institution which has been fortunate and has worked diligently to build a substantial inventory, two years ago began promoting legislation that would create short term federal funding to address this national need. Last year, legislation was introduced in the House (HR2852) by Representatives Smith, Burr, Davis, Towns, Doolittle, Toomey, Faleomavaega, Weldon, and Myrick. This was followed by a bill in the Senate (S1717) introduced by Senators Hatch, Brownback, Specter, Dodd, as well as Feinstein, and Jeffords.

These bills commit the Federal Government to establish funding through the Department of Health and Human Services (HHS) to qualified Cord Blood Banks in order to grow the nation’s cord blood inventory by 150,000 units. The essentials of the legislation represent the vision of the congressional sponsors and are as follows:

1. Funding will be sufficient to grow the nation’s supply of high quality, ethnically and geographically balanced, public cord bank blood units such that all persons may have access to a well-matched therapeutic unit if needed.
2. Cord blood banks from diverse areas of the US will be competitively selected based on defined criteria including demonstrated quality, population base, institutional commitment, etc. All units from the funded inventory will be accessible for search.
3. Selection of banks will be made by an ad hoc panel appointed by Health Resources Service Administration (HRSA) with Food and Drug Administration (FDA) playing a role in assessment and assurance of quality standards.
4. The HHS Secretary will appoint an independent Board of Directors composed of representatives of the Banks and other constituents. This board will oversee compliance with regulatory exigencies, set policy to maximize access to patients and advise the Secretary on matters pertaining to the use of the funds.
5. Banks will provide cord blood units for peer-reviewed research.
6. The public will be educated on the availability of cord blood and its therapeutic value.
7. Funding is primarily aimed at building the inventory and would go directly to banks for their operations. Banks would be expected to be self-sufficient through operations within five years with

continuing federal support ceasing at that time. Minor funds would be reserved for activities of the Board of Directors while it is in place.

A cornerstone of this legislative effort is recognition that cord blood banking, no longer experimental, is now a fully accountable activity that often has dire consequences when quality standards are not recognized or met. ***To assure product safety and efficacy, cord blood banking requires strict adherence to cGMP with at least the rigor of FDA regulated blood banking.*** The bills require banks to obtain “all applicable Federal and State license, certifications and registrations”, including registration with the Food and Drug Administration (FDA). With the expansion of cord blood banking activity and the diversity of experience and techniques used, ***anecdotal reports of distribution of cord blood units that have little or no therapeutic use have become disturbingly more common.*** When a patient is conditioned and treated with such a unit the outcome is often fatal. Cord blood practice and potential standards have now been reviewed by FDA through their IND process. To safeguard these most vulnerable patients, ***the nation must now take the next obvious step to regulate this critical activity through licensure by FDA.*** As it excels in regulating blood products for transfusion, this agency, through Federal Law is the only reliable way to assure minimum cord blood standards for the safety of patients.

The proposed legislation has already yielded important results including passage of a \$10M Appropriation in FY2004 and \$9.4M in the President's FY2005 budget. Senator Arlen Specter was instrumental in these appropriations. In the conference committee there was a difference of opinion over the “independent network concept”. Representative Bill Young, Chairman of the House Appropriations Committee, suggested setting aside funds for a study to be performed by the Institute of Medicine (IOM) in order to make recommendations on the optimal model for administering the funds and implementing the network. IOM will examine the merits of the intent of the legislation to have cord blood banks funded directly with administration through an independent Board of Directors versus assigning all responsibility and incorporating the network funding and administration into the National Marrow Donor Program (NMDP).

From our perspective, as initiators of these legislative efforts, the arguments for an independent network versus NMDP incorporation are as follows:

1. The bills introduced describe an ***enterprise model of independent cord blood banks without corporate connections or overriding operational bureaucracy.*** It is a lower cost model with no middleman adding costs, complexity or bureaucracy between the cord blood bank and the transplant center. This approach is ***more likely to drive innovation, efficiency, and improved patient outcomes - much as the nation's independent blood centers now provide well over half of our blood supply.*** Cord blood/cell therapy is a rapidly evolving activity. It will progress most effectively by maximizing necessary resources and minimizing bureaucracy and large institutional imperatives.
2. As emphasized above, quality standards review and enforcement should be decentralized and placed under federal law, preferably FDA. ***Reliance on administration of standards, accountability and control through a large central bureaucracy potentially introduces political bias and inefficiency that is unlikely to benefit patients.*** There are well known unfortunate examples of how large bureaucratic organizations have been unable to conform to minimum standards for biologics that the nation should not reproduce in cord blood or cell therapy.
3. ***Modern distributed informatics begs for a decentralized database, search and distribution system for cord blood.*** Most existing banks already have local databases that must be managed and updated in any case. Centralized search and distribution systems, while perhaps necessary for bone marrow donors, are neither necessary nor desirable for cord blood. A distributed database search algorithm would bypass the need for a duplicative centralized database. The use of message-oriented middleware would allow searches to be performed efficiently at the bank level and all bank results

collated into a single report, if needed. Access to both cord blood units and marrow donors could be accomplished simply through a collaborative website. ***Such a system capable of serving an entire network of cord blood banks was recently demonstrated at New York Blood Center.***

4. While the efforts of NMDP and other registries in bone marrow donor coordination are laudable, ***cord blood is a competing technology and should not be under the control of its competition.*** It is unwise for the nation to interrupt its evolution and to create a national monopoly for cord blood or cell therapy – particularly through an organization with little track record or previous interest. Cord blood evolves from a very different set of concepts and skill sets. Unlike the bone marrow donor program, donor recruitment and management are minor issues in cord blood. On the other hand, ***cord blood banks must manufacture with processing, storage and supply management driven by cGMP concepts, much as our nation's blood center operations.*** These are not skill sets or core competencies essential or paramount to a bone marrow program.

5. The advantages of ***cord blood banking that operates separately from the bone marrow program are well illustrated in the Japanese system in which the bank network is independent of the bone marrow organization.*** This system has shown remarkable growth and success in saving lives while assuring the best product – cord blood or marrow for each patient. It has now served over 1,300 patients (560 in the last year alone) with an inventory of 20,000 cord blood units.

This exciting new therapeutic has only just begun to realize its potential for saving lives. It is cell therapy in its largest scale and most important contribution thus far with much more to come. It is important to note that all the progress made thus far has been accomplished outside of the constraints of a large national organization. New York Blood Center has built an inventory of over 24,000 units and distributed over 1,500 life saving cord blood units for patients worldwide. The progress made and the knowledge gained through this experience all came through direct and reciprocal contact with transplant centers so those clinical outcomes, the most relevant method for quality control could be correlated with technical data. The precious knowledge and know-how gained in this experience has been and will continue to be liberally shared with cord blood bankers around the nation and the world. ***Recognizing the progress yet to be made and the best format to realize progress, New York Blood Center has neither intention nor desire to administratively control or dominate funding of any cord blood network resulting from this or any legislation.***

We arrive at a critical crossroads where the nation is poised to make the necessary investment to make this therapeutic marvel available to all who need it. Many more lives will be saved, as bone marrow transplantation is clearly limited while cord blood emerges as an exciting and successful alternative. All applaud the historic efforts of NMDP to match bone marrow donors with transplant recipients – a difficult task at least. However, there is an important distinction between the cord blood model proposed by the forward-looking congressional leaders and sponsors of the legislation, and the model proposed by NMDP. The former allows the field to progress unfettered from the resource constraints to build supply while also having the flexibility and autonomy of locally controlled operations. The latter links cord blood banks in business and programmatic relationships to a large centralized system designed to perform quite a different function and without significant experience or previous commitment to cord blood banking. As pioneers in cord blood banking and a founding member of America's Blood Centers, we believe this debate is important for blood centers and transfusion medicine. Mostly however, it is important for patients - those who are the least involved in the debate but the most affected.

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Other information about cord blood banking at New York Blood Center can be obtained at [www.nationalcordbloodprogram.org](http://www.nationalcordbloodprogram.org) or the blood center website [www.nybloodcenter.org](http://www.nybloodcenter.org).