



U.S. Department of Health and Human Services

**Fiscal Year 2012 Annual Progress Report on the C.W. Bill
Young Cell Transplantation Program and National Cord
Blood Inventory Program**

Executive Summary

The Stem Cell Therapeutic and Research Act of 2005, Public Law (P.L.) 109-129, as amended by P.L. 111-264 (section 379(a)(6) of the Public Health Service Act), includes the following requirement:

“The Secretary, acting through the Administrator of the Health Resources and Services Administration, shall submit to the Congress an annual report on the activities carried out under this section.”

This is an update to the report transmitted to Congress on January 11, 2013. That report included information through September 30, 2011. This report provides background information about the programs authorized by this law, summarizes the Health Resources and Services Administration’s efforts to implement the law, describes the structure and operation of each program, and provides statistical information about the status of the programs as of September 30, 2012 (end of fiscal year 2012).

During FY 2012, the Advisory Council on Blood Stem Cell Transplantation held two meetings and made six recommendations to the Secretary of Health and Human Services. The new recommendations focus on licensure of umbilical cord blood by the Food and Drug Administration. Highlights of this report include expansion of the pool of volunteer adult marrow donors to 10.5 million registered donors through the end of fiscal year 2012, improved survival rates for unrelated-donor transplantation, and increased access to transplantation for minority patients. Additionally, the National Cord Blood Inventory (NCBI) continues to grow in size and diversity with approximately 9,000 new units being added annually. Between fiscal years 2007 and 2012, more than 53,000 new units of cord blood were added to the NCBI. The value of this inventory is demonstrated by the increased utilization of these cord blood units relative to the total inventory of publicly available cord blood units.

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Legislative Language

This report is being provided to Congress as outlined in the Stem Cell Therapeutic and Research Act of 2005, Public Law (P.L.) 109-129, as amended by P.L. 111-264 (section 379(a)(6) of the Public Health Service Act).

“The Secretary, acting through the Administrator of the Health Resources and Services Administration, shall submit to the Congress...an annual report on the activities carried out under this section.”

Introduction

The Stem Cell Therapeutic and Research Act of 2005 (P.L. 109-129), signed on December 20, 2005, and reauthorized on October 8, 2010, as P.L. 111-264 (section 379(a)(6) of the Public Health Service Act), authorizes the C.W. Bill Young Cell Transplantation Program (program) as the successor to the National Bone Marrow Donor Registry. The program provides the infrastructure for facilitating blood stem cell transplants for patients with leukemia and other life-threatening blood disorders with cells from donors not related to the patients. The program also provides the infrastructure for collection of data on transplant outcomes.

The enactment of P.L. 109-129, and reauthorization through P.L. 111-264, was preceded by efforts to reauthorize P.L. 101-616, which established the National Bone Marrow Donor Registry and authorized federal support for the collection of high-quality, genetically diverse cord blood units. As legislative deliberations continued, nearly \$10 million was appropriated in fiscal year (FY) 2004 to establish a National Cord Blood Stem Cell Bank Program within the Health Resources and Services Administration (HRSA), and FY 2005 appropriations provided approximately \$10 million in additional funds for the National Cord Blood Stem Cell Bank Program. The Conference Report for the FY 2004 appropriations act required HRSA to contract with the Institute of Medicine (IOM) to “recommend an optimal structure and address pertinent issues to maximize the potential of cord blood technology.” The report, “Cord Blood: Establishing a National Hematopoietic Stem Cell Bank Program,” was published on April 14, 2005. The IOM recommended a program structure that, among other things, linked participating transplant centers with participating cord blood banks, collected data on the outcomes of cord blood transplants, and ensured data regarding banked cord blood units and outcomes of cord blood transplants were available to policy makers. This structure is similar to what was subsequently authorized by P.L. 109-129.

While the current scope of activities required of the program is similar to that of its predecessor, slight differences exist. Under current law the program can be administered through four infrastructure contracts rather than through a single contract, as was the case under the National Bone Marrow Donor Registry. Additionally, P.L. 109-129 provides for an expanded scope of data collection on blood stem cell transplant outcomes, to include both related- and unrelated-donor transplants. Furthermore, P.L. 109-129 also established a goal for the National Cord Blood Inventory (NCBI) to build a public inventory of at least 150,000 new, high-quality, and genetically diverse cord blood units. These units are to be made available for transplantation

through the program. Finally, the statute requires establishment of the Advisory Council on Blood Stem Cell Transplantation (Advisory Council) to provide recommendations to the Secretary of Health and Human Services (the Secretary) on activities related to these programs.

Implementation of the Program

HRSA's implementation of the Stem Cell Therapeutic and Research Act of 2005 was guided by three principles:

1. Ensuring a single point of access to cells from cord blood and bone marrow for patients and physicians;
2. Rapidly beginning collection of high-quality, genetically diverse cord blood units; and
3. Collecting complete and standardized data on blood stem cell transplant outcomes.

Informed by extensive consultation with transplantation experts during 2004 and 2005, HRSA began implementing all components of the program immediately after the legislation was enacted. HRSA issued competitive Requests for Proposals (RFPs) for all of the contracted infrastructure components of the program and for the NCBI in the spring of 2006. Contracts for the four infrastructure components of the program were awarded in September 2006, and NCBI contracts were awarded to six umbilical cord blood banks early in November of 2006. During 2006, HRSA also established the Advisory Council. HRSA kept Congress informed of its implementation approach through several briefings in 2006. The first meeting of the Advisory Council was held in January 2008.

HRSA created a website for the program (<http://bloodcell.transplant.hrsa.gov>) through which the public can get information about the C.W. Bill Young Cell Transplantation Program, the NCBI, and the Advisory Council. The website provides a variety of information for patients, physicians, and the public regarding blood stem cell transplantation and umbilical cord blood banking. Patients and providers can also use the website to perform a preliminary scan of the adult donor registry and the NCBI. This provides a preliminary estimate of the number of potentially suitable tissue-matched adult donors and umbilical cord blood units that are available through the program. Members of the public can also submit questions and comments through the website.

Subsequent sections of this report provide additional detail on the program, the NCBI, and the Advisory Council.

C.W. Bill Young Cell Transplantation Program Overview

As the successor to the National Bone Marrow Donor Registry, the C.W. Bill Young Cell Transplantation Program provides a structure to facilitate blood stem cell transplantation for patients with leukemia and other life-threatening blood disorders with blood forming cells from unrelated donors. The program also enables collection of data on the clinical outcomes of

transplants. The program is operated through a total of four major contracts that were competitively awarded in September 2006, each with a 2-year base period and four additional 1-year options.

Contracts for all components of the program were awarded through a competitive contracting process that emphasized technical merit. Contract opportunities were announced nationally, and proposals were evaluated by technical review committees composed of individuals with expertise in fields related to the program. Funding decisions were made based on committee assessments of technical merit, evaluation of past performance, and evaluation of proposed costs. When exercising option years beyond the original base period of the contracts, HRSA considers contractor performance and compliance with contract terms. (Figure 1 illustrates how the program was structured throughout FY 2012.) Following is a list of those contracts:

1. The Bone Marrow Coordinating Center coordinates a network of organizations to recruit, tissue-type, and manage potential donors of blood stem cells with an emphasis on the recruitment of individuals from diverse populations.¹ The Bone Marrow Coordinating Center also engages in public and professional education activities related to donation and transplantation.
2. The Cord Blood Coordinating Center coordinates a network of public cord blood banks and engages in public and professional education activities related to umbilical cord blood donation and transplantation.
3. A combined Single Point of Access and Office of Patient Advocacy maintain the single portal through which physicians and patients can identify and access a suitable blood stem cell product from an adult donor or cord blood unit. The Single Point of Access and Office of Patient Advocacy also provide supportive services to patients in need of a blood stem cell transplant from time of diagnosis through survivorship.
4. The Stem Cell Therapeutic Outcomes Database collects and analyzes data on the clinical outcomes of blood stem cell transplants. The Outcomes Database is also developing an approach to collect data on emerging therapeutic uses of cells found in umbilical cord blood, bone marrow, and peripheral blood (i.e., uses other than for hematopoietic reconstitution).

In FY 2012, the National Marrow Donor Program (NMDP) operated the Bone Marrow Coordinating Center, Cord Blood Coordinating Center, and a Single Point of Access and Office of Patient Advocacy contracts. The NMDP is a not-for-profit organization based in Minneapolis, Minnesota, and is the same organization that held a series of competitively awarded contracts for the National Bone Marrow Donor Registry for nearly 2 decades. The Center for International

¹ Sources of blood stem cells for unrelated-donor transplantation include marrow, peripheral blood stem cells, and umbilical cord blood. Donors of marrow and peripheral blood stem cells are both considered to be “adult donors” because the blood stem cells originate in the marrow of adult volunteer donors. The blood stem cells found in umbilical cord blood originate in the placenta and umbilical cord. All sources of blood stem cells are used to treat adult and pediatric patients.

Blood and Marrow Transplant Research (CIBMTR) at the Medical College of Wisconsin operated the Stem Cell Therapeutic Outcomes Database in FY 2012. With support from the National Institutes of Health and HRSA, the CIBMTR has been the principal organization in the United States collecting and analyzing data on clinical outcomes of blood stem cell transplants over the past 3 decades.

The funding history for the program contracts through FY 2012 can be found in Table 1 (the funding history for the NCBI contracts through FY 2012 can be found in Table 3).

Figure 1: C.W. Bill Young Cell Transplantation Program

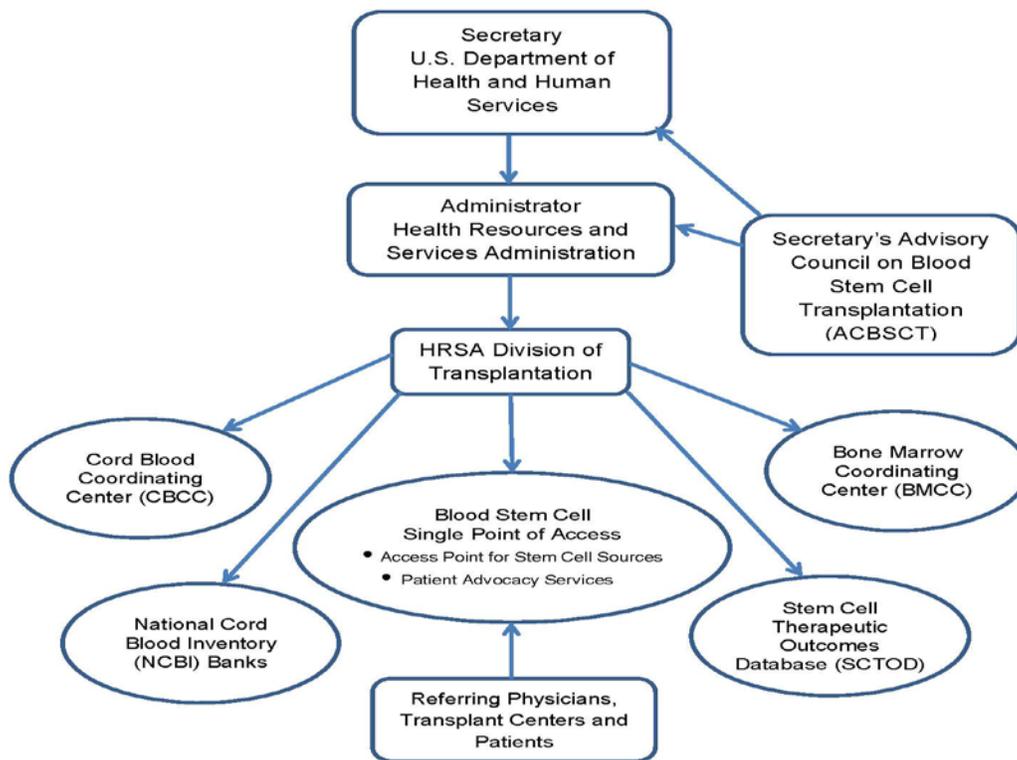


Figure 1: The C.W. Bill Young Cell Transplantation Program is comprised of the Cord Blood Coordinating Center, the Bone Marrow Coordinating Center, the Stem Cell Therapeutic Outcomes Database, and the Blood Stem Cell Single Point of Access. Patients and health care providers access blood stem cell products and receive donor advocacy services working through the Blood Stem Cell Single Point of Access. The oval labeled “National Cord Blood Inventory Banks” represents the 13 public umbilical cord blood banks under contract to HRSA for the NCBI. The cord blood banks collect, test, and distribute cord blood units for transplantation through the Cord Blood Coordinating Center. The Secretary’s Advisory Council on Blood Stem Cell Transplantation makes recommendations to the Secretary and to the Administrator, HRSA, on matters carried out by the program. HRSA’s Healthcare Systems Bureau, Division of Transplantation is the operating division responsible for providing oversight of the C.W. Bill Young Cell Transplantation and NCBI Programs.

Table 1: Allocations for the Program Contracts, FY 2006 – 2012

Fiscal Year	Appropriation+	Bone Marrow Coordinating Center*	Cord Blood Coordinating Center*	Single Point of Access and Office of Patient Advocacy*	Stem Cell Therapeutic Outcomes Database	Total Obligation to Program Contracts
2006	\$25,145,000	\$16,125,221	\$1,850,985	\$1,226,354	\$4,000,000	\$23,202,560
2007	\$25,168,000	\$21,125,660	\$0	\$0	\$2,240,629	\$23,366,289
2008	\$23,517,000	\$16,193,085	\$993,886	\$640,637	\$3,684,928	\$21,512,536
2009	\$23,517,000	\$16,131,600	\$1,045,867	\$659,928	\$3,663,174	\$21,500,569
2010	\$23,517,000	\$15,671,402	\$1,196,701	\$679,991	\$3,883,247	\$21,431,341
2011	\$23,374,000	\$15,640,305	\$749,834	\$700,855	\$4,189,900	\$21,280,894
2012	\$23,330,000	\$16,048,000	\$654,990	\$705,505	\$3,847,741	\$21,256,236
Total	\$167,568,000	\$116,935,273	\$6,492,263	\$4,613,270	\$25,509,619	\$153,550,425

+Appropriation amounts after rescission and Secretary's transfer.

*These are cost-sharing contracts. The amount provided reflects the government's share.

Professional and Public Education Activities

In FY 2012, the contractors for the C.W. Bill Young Cell Transplantation Program (i.e., the NMDP and the CIBMTR) continued to deliver valuable educational resources and services to the physicians who refer patients for blood stem cell transplantation and those who treat patients with transplantation, as well as the general public. The NMDP carried out several initiatives to engage health professionals about the importance of timely transplant planning to improve the likelihood of patients progressing to transplant, when needed. The NMDP also continued its Continuing Medical Education programs to focus on increasing awareness of appropriate transplant indications, including Myelodysplastic syndromes (MDS) and sickle cell anemia. Prior to 2010, many patients diagnosed with MDS did not have access to hematopoietic cell transplantation because of lack of reimbursement for the procedure by Medicare. In recent years, the number of patients who have received allogeneic hematopoietic cell transplantation for treatment of MDS has continued to grow through a research study conducted by CIBMTR and approved under Coverage with Evidence Development by Centers for Medicare and Medicaid Services (CMS). This study will provide CMS with evidence regarding the effectiveness of hematopoietic cell transplantation in treating MDS to guide future policy decisions. At the end of FY 2012, 111 U.S. transplant centers had enrolled patients on this study (see Table 2 below for number of patients by FY).

Table 2: Number of patients 65 years of age or older registered with the CIBMTR that received an allogeneic transplant at a U.S. center for MDS in fiscal years 2010 through 2012.

Fiscal Year	Related Donor	Unrelated Donor	All
2010	44	61	105
2011	43	91	134
2012	68	146	214

Other professional education and outreach efforts included the development of programs and materials to update transplant physicians on the donor-recipient tissue matching guidelines and the latest trends in unrelated-donor and umbilical cord blood transplant research. Finally, the NMDP developed post-transplant care plans that referring physicians can use when their patients return to their care after having undergone transplantation.

Public outreach and education activities undertaken by the NMDP in FY 2012 include targeted recruitment on college campuses with an emphasis on Historically Black Colleges and Universities as well as Hispanic Serving Institutions. The NMDP embraced social media as a way of increasing visibility and public awareness about registering to become a volunteer marrow donor. Finally, the NMDP spent the year focusing on engaging families of current patients with the intent of increasing the number of individuals listed on the adult donor registry. Family members are important advocates and partners in the effort to grow the unrelated-donor registry.

Transplant Survival Rates

The program had a long-term goal of increasing the 1-year survival rate of unrelated-donor blood stem cell transplants from 62 percent in FY 2003 to 69 percent in FY 2010. To achieve this goal, the program, working through its contractors:

1. engaged in education efforts about the appropriate timing for referral to transplant and those resources available to transplant candidates and their providers;
2. provided additional training to transplant centers about best practices for donor-recipient tissue matching and educated them about the various sources of blood stem cells available to them (i.e., adult donors and cord blood);
3. worked to improve the speed at which donor searches can be performed, which allows more patients to proceed to transplant at the clinically optimal time;
4. used data submitted to the Stem Cell Therapeutic Outcomes Database to refine transplant protocols and patient selection to better determine which patients will benefit from transplant; developed tools to support transplant patients as they transition into long-term survivorship; and

5. grew the number of adult volunteer donors and cord blood units available through the program to optimize the number of patients who are able to receive appropriately matched blood stem cells.

As a result of this work, the survival rate of unrelated-donor transplant recipients has increased at a rate of about 1.5 percent per year since 1990. Given the amount of time it takes to track patients to 1-year post-transplant, FY 2010 survival rate data were not available at the end of 2012 (and will be provided in the next report).

National Cord Blood Inventory (NCBI) Overview

The goal of the NCBI Program is to build a genetically diverse inventory of at least 150,000 new units of high-quality umbilical cord blood. These units are to be made available to patients through the C.W. Bill Young Cell Transplantation Program. Donated units that are not suitable for clinical transplant are made available for peer-reviewed research. The ability to achieve the goal of at least 150,000 new units of cord blood depends on the availability of funds. It costs the cord blood banks between \$1,500 and \$2,500 to collect, test, and cryopreserve each cord blood unit they make available for listing through the C.W. Bill Young Cell Transplantation Program.

HRSA awards contracts to public cord blood banks through the competitive RFP process for collection, testing, banking, and distributing cord blood units. Successful cord blood banks are reimbursed on a per unit basis for each unit that meets all of the criteria specified in their contracts. The contracts specify total number of units to be reimbursed per year as well as the racial/ethnic breakdown of those units. Setting goals for the racial/ethnic breakdown of the units helps ensure continued growth in the diversity of tissue types available for transplantation. This is critical for those populations that typically have a difficult time finding a suitably matched adult blood stem cell donor.

The 2005 authorizing legislation stipulated that NCBI contractors could initially receive no more than 3 years of funding, although contract modifications to extend the period of funding beyond 3 years were allowable under certain circumstances specified in the legislation. Although funding was limited to 3 years, contractors were obligated to participate in the C.W. Bill Young Cell Transplantation Program for 10 years. With the 2010 reauthorization, Sections 2(e)(1) and 2(e)(2) of the statute allow for up to 5 years of funding with the possibility of a contract modification to provide funding for an additional 5 years. Additionally, the reauthorization extends the amount of time contractors are required to participate in the program to 10 years from the date on which they last received NCBI funds. HRSA ensured that the competition for award of new contracts under the program was open to new banks interested in collecting, testing, and banking cord blood units.

HRSA conducts annual reviews of each contractor's progress. Subject to the availability of funds, options to support the banking of additional cord blood units are exercised for contractors who have demonstrated the ability to meet the program's goals as identified by the authorizing statute (i.e., the ability to collect and store diverse, high-quality cord blood units for unrelated-donor transplantation). Funding decisions have aimed to ensure that progress is made toward achieving the goal of banking at least 150,000 new units of cord blood while ensuring continued

growth in the diversity of the available inventory. Table 3 shows the appropriation and funding history of the NCBI through FY 2012.

Table 3: Appropriations and Contract Allocation History for the NCBI through FY 2012

Fiscal Year	Appropriation+	Total Contract Award Amount	Number of NCBI Cord Blood Units Purchased
2004 – 2007*	\$27,720,000	\$25,898,929	23,049
2008	\$8,843,000	\$8,287,707	8,938
2009	\$11,983,000	\$11,240,309	10,207
2010	\$11,983,000	\$11,500,295	9,900
2011	\$11,910,000	\$11,384,224	10,571
2012	\$11,887,000	\$10,976,299	9,105
Total	\$84,326,000	\$79,287,763	71,770

+ Appropriation amounts after rescission and Secretary’s transfer.

*Contract awards during FY 2007 used funds from no year appropriations for FYs 2004-2006 and annual appropriations for FY 2007. Per the Appropriation Act for FY 2004, \$988,190 was awarded for a contract with the IOM to recommend a structure for the National Cord Blood Stem Cell Bank Program.

HRSA has awarded 18 NCBI contracts since the inception of the program through FY 2012, (five contractors hold two contracts each), and has provided funds to store over 71,000 NCBI units, with over 60 percent from minority donors. Figure 2 identifies organizations holding an NCBI contract as of the end of FY 2012. The figure also shows the geographic distribution of NCBI contractors. A good geographic dispersion not only ensures continued availability of cord blood units should a disaster temporarily impact one region of the country, but it also helps ensure that as diverse a group of donors as possible are given the opportunity to donate cord blood.

Figure 2: Geographical Distribution of NCBI Banks



Figure 2: As of the end of FY 2012, HRSA has contracted with 13 umbilical cord blood banks to collect, test, store, and distribute cord blood units for the NCBI. Those contractors are: Carolinas Cord Blood Bank at Duke University (Duke University), Cleveland Cord Blood Center (Cleveland CB Center), CordUSE Cord Blood Bank (CordUSE), JP McCarthy Cord Stem Cell Bank at Wayne State University (JP McCarthy), LifeCord Cord Blood Bank at LifeSouth Community Blood Centers (LifeCord), New Jersey Cord Blood Bank at Bergen Community Regional Blood Center (New Jersey CBB), New York Blood Center, Puget Sound Blood Center, St. Louis Cord Blood Bank at SSM Cardinal Glennon Children’s Medical Center (St. Louis CBB), South Texas Blood and Tissue Center (South Texas CBB), StemCyte, Inc. (StemCyte), University of Colorado, and University of Texas MD Anderson Cancer Center (MD Anderson).

Cord Blood Demonstration Project

The Cord Blood Demonstration Project to implement a Remote Kit-Based Collection Pilot Project was conducted from October 1, 2009, through September 30, 2012. One of the goals of the project was to expand the opportunity to donate umbilical cord blood to more families. HRSA conducted the pilot through the Cord Blood Coordinating Center to determine the feasibility and utility of decentralized umbilical cord blood collections (i.e., collection of cord blood units at hospitals that are not staffed or directly supported by a cord blood bank). As part of the pilot, the Cord Blood Coordinating Center and three public cord blood banks promoted cord blood donation through informational pamphlets, radio advertisements, and print ads. Interested families were directed to contact the Cord Blood Coordinating Center or the cord blood banks for more information or to request a collection kit. Collection kits were mailed to those potential donor mothers meeting the initial eligibility criteria. The eligible participants took the collection kits with them to their birthing hospitals at the time of labor. The providers

were trained on how to collect the umbilical cord blood remotely. After the baby's birth and collection of his or her umbilical cord blood, the cord blood unit was transported to one of the three participating banks using validated shipping methods.

Cord Blood Demonstration Project Results

Throughout the entire 3 years of the pilot, maternal donor enthusiasm and interest in donating via a kit-based collection approach was high. The pilot offered increased opportunities to expectant mothers who would not otherwise be able to donate their umbilical cord blood because there was no collection hospital in their area. Although the goal of the pilot was to collect 500 cord blood units through this pilot, the actual number of units collected was 252. The pilot was also projected to have a cord blood collection to cord blood banking ratio of 25 percent; however, the actual banking goal was 13 percent. According to the three NCBI banks that participated in the pilot, the primary two reasons for not meeting these established goals were low blood volumes in the umbilical cord blood units (34 percent of donated cord blood units) and the time it took from collecting the umbilical cord blood units to transporting them to the cord blood bank for processing within the 8 hour window (26 percent of donated cord blood units). The pilot resulted in increased awareness from racial and ethnically diverse populations. A total of 39 percent of the cord blood donors self-identified as belonging to an underrepresented racial or ethnic minority group.

Next steps

The pilot resulted in creating opportunities for cord blood collections for expectant mothers who would otherwise not be able to donate their babies' umbilical cord blood. The C.W. Bill Young Cell Transplantation Program plans to continue to make remote collection opportunities available to expectant mothers in the future. Plans are underway to identify ways to overcome the barriers revealed in the pilot (e.g., improve the shipping times from cord blood collection to processing and provide enhanced training for obstetricians to possibly improve the blood yields in the umbilical cord blood).

The 2010 reauthorization of the C.W. Bill Young Cell Transplantation Program requires the Secretary in Section 379 (d)(2)(B) of the Public Health Service Act to annually identify a project that focuses on increasing cord blood unit donation and collections from diverse populations and increasing the number of hospitals where umbilical cord blood can be donated. The project must be replicated and expanded nationwide. HRSA worked in 2012 through the Cord Blood Coordinating Center to implement a project that expanded upon the Cord Blood Demonstration Project. The project focused on the development of a web-based "Learn if you can donate" tool to assist cord blood banks in their request for centralized assistance in screening and providing information to expectant mothers. The tool was designed to help mothers easily determine if they meet basic cord blood donation guidelines, provide information about the donation process, and connect them to a cord blood bank for further evaluation if the initial screening is acceptable. The goal of the tool was to decrease the time cord blood banks spend with expectant mothers at that first entry point. Additional details on the implementation of the "Learn if you can donate tool" will be provided in the FY 2013 Report to Congress.

Program and NCBI Statistics

- Transplants facilitated by the program grew from 2,519 in FY 2004 to 5,833 in FY 2012 (an increase of 131 percent).
 - 1,150 of the transplants used marrow products.
 - 3,492 of the transplants used peripheral blood stem cells.
 - 1,191 of the transplants used umbilical cord blood.

- Transplants for minority patients have grown at a faster rate than for non-minority patients. 394 transplants for minority patients were facilitated by the program in FY 2004 and 1,021 were facilitated by the program in FY 2012 (an increase of 159 percent).
 - Transplants for minorities that utilized adult donors as the source of blood stem cells grew from 542 in FY 2011 to 624 in FY 2012 (an increase of 15.1 percent).
 - The number of transplants for minority patients increased from 955 in FY 2011 to 1,021 in FY 2012 (an increase of 6.9 percent). Transplants for minority patients represent 17.5 percent of the total number of transplants facilitated by the program in FY 2012.
 - Transplants for Black or African-American patients increased from 276 in FY 2011 to 310 in FY 2012 (an increase of 12.3 percent).
 - Transplants for Asian patients increased from 202 in FY 2011 to 212 in FY 2012 (an increase of 5 percent).
 - Transplants for Hispanic or Latino patients increased from 443 in FY 2011 to 459 in FY 2012 (an increase of 3.6 percent).

- Growth in the availability of umbilical cord blood units has greatly extended access to transplant, particularly for minority patients.
 - Approximately 33 percent of all cord blood transplants performed in FY 2012 were for minority patients. In contrast, only 13 percent of transplants utilizing blood stem cells from adult donors were for minority patients in FY 2012.
 - Minority cord blood transplants dropped from 413 in FY 2011 to 397 in FY 2012 (a decrease of 4 percent).
 - The number of Black or African-American patients who received a cord blood transplant in FY 2012 increased 2.2 percent over FY 2011.
 - The number of Asian patients who received a cord blood transplant in FY 2012 decreased 12.3 percent over FY 2011.
 - The number of Hispanic or Latino patients who received a cord blood transplant in FY 2012 decreased 6.7 percent over FY 2011.
 - A greater percent of minority patients who received a transplant in FY 2012 received cord blood as compared to all patients who received a transplant.
 - Of the minority patients who received a transplant in FY 2012, nearly 39 percent received cord blood as their source of blood stem cells.
 - Among all patients who received a transplant in FY 2012, only 20 percent received cord blood as their source of blood stem cells.

- Approximately 10.6 million potential donors, 27 percent of whom are from minority populations, were included in the program registry as of September 30, 2012.
 - In FY 2012, a total of 508,716 potential adult donors of bone marrow or peripheral blood stem cells were added to the registry.
 - 240,657 (47 percent) of these potential adult donors self-identified as belonging to an underrepresented racial or ethnic minority group.
- Over 184,000 cord blood units were available through the program as of September 30, 2012. This includes cord blood units made available through the program by domestic and international umbilical cord blood banks. Additionally, a subset of these cord blood units was funded through the NCBI.²
 - As of September 30, 2012, 53,609 NCBI cord blood units are available to patients through the program.³
 - This represents an increase of 10,269 (23.7 percent) cord blood units since the last report to Congress.
 - 60 percent of these units are from minority donors.
 - Approximately 18,161 more units await listing or will be collected with funds awarded to cord blood banks through the end of FY 2012.
 - In FY 2012, a total of 714 NCBI cord blood units were released for transplantation bringing the total number of units released for transplantation, since 2007, to 2,450 units.
 - In FY 2007, 4 NCBI cord blood units were released for transplantation.
 - In FY 2008, 104 NCBI cord blood units were released for transplantation.
 - In FY 2009, 408 NCBI cord blood units were released for transplantation.
 - In FY 2010, 530 NCBI cord blood units were released for transplantation.
 - In FY 2011, 690 NCBI cord blood units were released for transplantation.
- As of September 30, 2012, more than 26,000 cord blood units not meeting criteria for clinical transplantation have been made available for research purposes.
- In FY 2012, 1,436 related donor-recipient research specimens were submitted to the repository maintained by the CIBMTR, contractor for the Stem Cell Therapeutic Outcomes Database. The NMDP also maintains an unrelated donor-recipient sample repository. 10,303 specimens were submitted to this in FY 2012.
- In FY 2012, the CIBMTR published 54 peer-reviewed publications on blood stem cell transplantation. The studies were published in several journals including: *Blood*, *New England Journal of Medicine*; and, *Biology of Blood and Marrow Transplantation*. A list of (CIBMTR) 2012 publications are posted on: <http://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx?year=2012>

² The total number of cord blood units available through the program includes cord blood units funded through the NCBI as well as other non-NCBI cord blood units meeting quality and safety criteria for use in blood stem cell transplantation.

³ The NCBI cord blood units are a subset of the total inventory of public cord blood units available through the C.W. Bill Young Cell Transplantation Program.

Advisory Council on Blood Stem Cell Transplantation

In accordance with the Stem Cell Therapeutic and Research Act of 2005, Public Law (P.L.) 109-129, as amended by P.L. 111-264 (section 379(a)(1)), the Secretary has also established an Advisory Council. The Advisory Council advises the Secretary and the HRSA Administrator on matters related to the C.W. Bill Young Cell Transplantation Program and the NCBI. By the end of FY 2012, the Advisory Council had held 11 meetings.

Since its inception, the Advisory Council has formed working groups to explore matters related to the following topics:

- Umbilical cord blood bank accreditation;
- Public funding for required outcomes data;
- Cord blood donor confidentiality;
- Access to cord blood units for research;
- Informed consent for cord blood donation;
- The scientific factors defining a high-quality cord blood unit;
- Gaps in insurance coverage for transplant;
- Maximizing cord blood collections;
- Procedures for preparing cord blood units for infusion; and
- Maximizing the potential uses of cord blood.

By the end of FY 2012, 22 recommendations had been submitted to the Secretary. All Advisory Council meetings are open to the public and announced in the *Federal Register*. Each meeting provides attendees an opportunity to make public comment. The charter for the Advisory Council, membership roster, agenda for upcoming meetings, and meeting summaries are available on the C.W. Bill Young Cell Transplantation Program website <http://bloodcell.transplant.hrsa.gov>. Table 4 provides a summary of the 22 recommendations submitted to the Secretary as of September 30, 2012.

Table 4: Advisory Council Recommendations.

Meeting Date	Summary of Recommendation	Status of Recommendation
April 29, 2008	<p>1. The Advisory Council recommended that the Food and Drug Administration (FDA) finalize its Draft Guidance for Industry on Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood intended for Hematopoietic Reconstitution in Patients with Hematological Malignancies, and in so doing broaden the clinical indications covered by a license, provide a path to licensure for high quality units already in the inventory, and provide for the import of high quality cord blood units.</p>	<p>The FDA issued a guidance, “Minimally manipulated, unrelated allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for specified Indications” on October 20, 2009, which addresses the Advisory Council’s stated concerns. At the same time, the FDA issued a draft guidance advising entities, including transplant centers and individual physicians draft guidance advising entities, including transplant centers and individual physicians on filing Investigational New Drug application (IND) to access unlicensed cord blood units when a suitable human leukocyte antigen (HLA) matched cord blood transplant is needed for treatment of a patient with a serious or life-threatening disease or condition and there is no satisfactory alternative treatment available. The FDA has since revised that final guidance and finalized that draft IND guidance. The most current versions were issued in March 2014.</p>
	<p>2. The Advisory Council recommended that the Secretary restore full funding of the CIBMTR research focused cooperative agreement with the National Cancer Institute.</p>	<p>The National Institutes of Health provided additional funding to support the CIBMTR’s research focused cooperative agreement. The cooperative agreement had a project period of March 1, 2008-February 28, 2013. The cooperative agreement was renewed on March 1, 2013 and will run through February 28, 2018.</p>
December 16, 2008	<p>3. The Advisory Council recommended that the Secretary direct CMS, as a high priority, to develop an appropriate strategy for a National Coverage Determination on allogeneic stem cell transplantation as therapy for MDS based on the recent evidence-based review of the literature.</p>	<p>On August 4, 2010, CMS concluded that additional research from clinical trials would be appropriate under the Coverage with Evidence Development (CED) aspect of CMS’ coverage authority. CMS issued a national coverage decision allowing Medicare coverage of allogeneic hematopoietic stem cell transplant for the treatment of MDS when provided to Medicare beneficiaries enrolled in an approved clinical study. On December 15, 2010, CMS approved a study, which was submitted by the CIBMTR for Medicare patients with MDS as eligible for CED.</p>

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	4. The Advisory Council recommended that the C.W. Bill Young Cell Transplantation Program implement policies that conform to a number of specific principles to protect the privacy and confidentiality of cord blood donors donating umbilical cord blood units to the program.	In FY 2010, HRSA reviewed its contract specifications for the C.W. Bill Young Cell Transplantation Program and the National Cord Blood Inventory related to privacy and confidentiality requirements for cord blood donors and adult marrow donors and determined that its requirements are aligned with the principles the Advisory Council identified.
May 12, 2009	5. The Advisory Council recommended that the Secretary recognize both the American Association of Blood Banks (AABB) and the Foundation for the Accreditation of Cellular Therapy (FACT) as accreditation organizations for the NCBI. Both organizations are expected to adhere to HRSA's specifications for accreditation organizations, and their continued recognition is based on ongoing adherence. The Advisory Council will review, 3 years from the time of the recognition decision by the Secretary, HRSA's experience with the accreditation organizations with regard to meeting HRSA's specifications.	HRSA worked to develop Memoranda of Understanding (MOU) with two accrediting organizations. In FY 2012, HRSA signed a MOU with AABB and FACT allowing the organizations to conduct cord blood bank assessments against HRSA-specific criteria.
September 21, 2009	6. The Advisory Council recommended informed consent principles and standards for public cord blood banking.	In August 2009, HRSA reviewed its NCBI contracts to ensure consistency with the Advisory Council's recommendations. HRSA determined that its existing language complied with the Advisory Council's recommendation.

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May 5, 2010	7. The Advisory Council recommended that an expert panel be convened to review and recommend clinical indications for which stem cell transplantation is covered by insurance plans.	At its May 5, 2010, meeting, the Advisory Council charged one of its workgroups to continue to explore this recommendation and to develop potential options. The blood stem cell community leveraged the work of the NMDP, and participated in its Advisory Group on Financial Barriers to Transplantation to address inadequate transplant coverage. HRSA has ex-officio membership on this Advisory Group and is actively involved in its efforts.
	8. The Advisory Council recommended that the Secretary mandate that Medicare and Medicaid cover patient participation in clinical trials involving hematopoietic transplantation.	HRSA and its contractor, the NMDP, continue to work with CMS on a case by case basis to provide model benefits language for clinical trials involving hematopoietic transplantation.
	9. The Advisory Council recommended several ways in which cord blood collections could be improved and increased.	HRSA reviewed this recommendation with public cord blood banks participating through the NCBI program, and it has been adopted where applicable. HRSA and NMDP have worked with the public cord blood bank community to identify ways to increase and improve cord blood collection. For example, in FYs 2013 and 2014, HRSA provided additional resources to federally-funded NCBI cord blood banks to increase the number of cord blood collections at new or existing collection centers.
November 15, 2010	10. The Advisory Council recommended that the Secretary recognize hematopoietic transplantation for generally accepted indications as a covered benefit for all federal programs for which the Secretary has appropriate responsibility and oversight.	HRSA and NMDP are working with the American Society of Bone and Marrow Transplantation to complete a list of indications for transplant, which represent the current standard of care. This list of proposed transplant indications will inform future discussions on potential changes to coverage by federal programs. In addition, the NMDP, through its Advisory Group on Financial Barriers to Transplant, has developed model benefits language to share with public and private insurers and benefits managers. HRSA has ex-officio membership on this Advisory Group and is actively involved in its efforts.

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	11. The Advisory Council recommended to the Secretary that Medicare reimburse for the acquisition of blood, marrow and cord blood products for hematopoietic transplantation on a cost basis similar to how reimbursement is made for graft acquisition in solid organ transplantation.	Discussions between HRSA and CMS are ongoing on this issue.
	12. The Advisory Council recommended that the Secretary clarify that the expiration date can be placed on an attached label provided with the unit at time of release to a transplant center for cord blood units incapable of bearing a full label.	Through the FDA licensure process, which has resulted in five HRSA-funded NCBI banks obtaining licenses to date (starting November 2011), the FDA has clarified guidance on labeling to permit attachment of a tag disclosing expiration date and other information as acceptable.
	13. The Advisory Council recommended that the Secretary work with the FDA to review requirements for licensure in light of concerns over the potential for licensure requirements to result in increased cost and decreased availability of public cord blood units, with the goal that FDA urgently meet with the cohort of applicant cord blood banks and representatives of transplant centers to share and resolve specific concerns regarding licensure.	The FDA has engaged in a number of outreach efforts to educate public cord blood banks about the licensure process and to provide technical assistance, and those efforts are continuing. The FDA has ongoing conversations with the cord blood banking and transplant center community and participates in industry conferences. The FDA has also held meetings with individual cord blood banks upon request, in order to assist them with meeting the licensure requirements and submission of a biological license application. The FDA recognizes that in some cases, the best matched cord blood unit for treating a patient with a serious or life-threatening disease may not be met by licensure requirements, and there may be no satisfactory alternative treatment available. Clinical use of such units is allowed under the IND pathway. The FDA's most current guidance on IND submissions for this purpose was issued in March 2014 as "Guidance for Industry and FDA Staff: IND Applications for Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic and Immunologic Reconstitution in Patients with Disorders Affecting the Hematopoietic System." http://www.fda.gov/biologicsbloodvaccines/guidancecomplianceregulatoryinformation/guidances/cellularandgenetherapy/ucm388218.htm

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	<p>14. The Advisory Council recommended to the Secretary that models for remote collection of cord blood units be allowed with only limited, scientifically justified safety precautions. The Advisory Council also recommended that the Secretary allow for cord blood unit collection from routine deliveries without temperature or humidity monitoring of delivery rooms in hospitals approved by the appropriate bodies for hospital accreditation.</p>	<p>HRSA implemented a pilot program to test whether remote cord blood collections can be performed in a safe and efficient manner. The pilot project was conducted from October 1, 2009, through September 30, 2012. The pilot's results showed that (1) the pilot offered increased opportunities to expectant mothers who would not otherwise be able to donate their umbilical cord blood, because there was no collection hospital in their area; (2) cord blood units can be collected remotely in a manner that is safe and efficient; and (3) the pilot resulted in increased awareness from racial and ethnically diverse populations. A total of 39 percent of the cord blood donors self-identified as belonging to an underrepresented racial or ethnic minority group.</p> <p>Even though the pilot has ended, a few public banks continue to offer this service. A number of banks, including some FDA licensed banks, now use remote collection to supplement their collection activity.</p>
<p>May 11, 2011</p>	<p>15. The Advisory Council recommended that the Secretary recognize public cord blood bank oversight of the collection process as sufficient means to ensure safe manufacturing practices and oppose the requirement for hospitals to register with the FDA as the establishment responsible for recovery.</p>	<p>In December 2011, the FDA communicated that, in general, recovery establishments are required to register and list with FDA (21 CFR Part 1271) unless an exception applies. Section 1271.15(f) provides that an individual under contract agreement or other arrangement with a registered establishment, and engaged in solely recovering cells or tissues for the registered establishment, may be exempt from requirements to register and list with the FDA, but must comply with all other applicable requirements in 21 CFR Part 1271.</p> <p>Individuals collecting cord blood at hospitals may fall within this exception if they are under contract, agreement or other arrangement with a registered establishment, including a cord blood bank.</p>

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	16. The Advisory Council recommended that the Secretary support the collection of cord blood from uncomplicated deliveries in accredited hospitals without environmental monitoring of delivery rooms.	In December 2011, the FDA published its “Guidance for Industry: Current Good Tissue Practice (CGTP) and Additional Requirements for Manufacturers of Human Cells, Tissues, and Cellular and Tissue-Based Products (HCT/Ps),” (see Section IX. H). ⁴ This guidance provided information about what environmental control and monitoring issues should be considered for recovery of HCT/Ps.
November 8, 2011	17. The Advisory Council recommended to the Secretary that the FDA recognize and accept laboratory-developed tests (LDTs) performed in Clinical Laboratory Improvement Amendment (CLIA)-certified, high complexity histocompatibility laboratories.	This issue has been discussed at the HHS-Interagency Workgroup on Hematopoietic Stem Cell Transplantation. The FDA published a “Proposed LDT Regulatory Oversight Framework” on September 30, 2014. ⁵ The framework is available for review and public comment. The FDA is specifically seeking feedback on LDTs used for rare diseases and traditional LDTs interpreted by laboratory professionals who are appropriately qualified and trained as required by the CLIA regulations, among other issues. While this policy is evolving, the FDA has been engaged and aware of the concerns of the blood stem cell and organ transplant communities with respect to HLA testing.
	18. The Advisory Council recommended that FDA broaden IND and biologics license application (BLA) clinical indications for unrelated-donor cord blood transplantation to include use for hematopoietic and/or immune reconstitution or enzyme replacement in any situation where hematopoietic stem cell transplantation is the appropriate approach to treatment.	In November 2011, the FDA granted the first license for a cord blood product. The product was licensed for use in patients with disorders affecting the hematopoietic system. Subsequently licensed cord blood products have had similar indications.

⁴ This can be found at <http://www.fda.gov/downloads/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/Tissue/UCM285223.pdf>.

⁵ This can be found at <http://www.fda.gov/MedicalDevices/ProductsandMedicalProcedures/InVitroDiagnostics/ucm407296.htm>.

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	19. The Advisory Council recommended that all cord blood products have the same IND and BLA clinical indications; this is scientifically and medically sound.	In March 2014, the FDA revised its language for covered indications. The titles of both FDA guidance documents now correspond to each other: (...intended for hematopoietic and immunologic reconstitution in patients with disorders affecting the hematopoietic system), and thus they are “the same” per the recommendation.
	20. The Advisory Council recommended implementation of a transition plan, initially stated as on or before October 20, 2011, to allow time for the FDA to review recommendations 17 and 18 above and to allow for implementation of any changes by transplant centers that will need to put these changes through their institutional review boards.	The NMDP has developed an IND protocol under which non-licensed cord blood units may be accessed and distributed. The FDA continues to work with other sponsors to address the availability of cord blood products.
	21. The Advisory Council recommended that cord blood units collected through distribution of kits sent to motivated maternal donors or obstetrical units collected by an obstetric provider, which meet all National Cord Blood Inventory/FDA qualifications, be eligible for listing on the NCBI and for FDA licensure.	In May 2011, the FDA confirmed that public cord blood banks, including those that hold NCBI contracts with HRSA, may apply for a BLA that covers remote kit collections. If FDA licensure is granted to NCBI contracted cord blood banks with the remote kit option included, cord blood units collected in this way may be listed and included in the NCBI. In May 2012, the first NCBI cord blood bank submitted an application with cord blood being collected through the distribution of kits. The FDA approved the collection model and those units are eligible for NCBI funding and listing.
May 9, 2012	22. The Advisory Council recommended that the Secretary take and support all reasonable efforts to ensure that compensation for marrow, peripheral blood stem cells and similar products continues to be prohibited.	On October 2, 2013, HRSA published a Notice of Public Rule-making in the Federal Register. Responses are still under internal HRSA review.

Conclusion

This report reflects the status of the C.W. Bill Young Cell Transplantation Program and the National Cord Blood Inventory Program as of September 30, 2012. It provides information on contracts awarded and funding, the adult donor registry, the cord blood inventory, patient transplant statistics, pilot and demonstration projects implemented, and recommendations made by the Advisory Council on Blood Stem Cell Transplantation. Information on progress and plans made will be reflected in future annual Congressional reports.