U.S. Department of Health and Human Services

HRSA

Health Resources and Services Administration

Fiscal Year 2014 Annual Progress Report on the C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory Program

Health Resources and Services Administration
Rockville, MD 20857

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 a requirement in Section 3 that:

> *"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 fiscal year (FY) 2013 annual report to Congress, which included information through September 30, 2013. This report provides background information about the programs authorized by the 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, 2014.

Since the development of the previous report, the Advisory Council on Blood Stem Cell Transplantation held two meetings and made two recommendations to the Secretary of Health and Human Services. The new recommendations address sickle cell disease educational and outreach efforts for the community.

Highlights of this report include an expanded pool of 12.4 million registered volunteer adult marrow donors, 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 over 7,000 new cord blood units being added each year. Between FYs 2007 and 2014, over 74,000 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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Acronym List

AABB	American Association of Blood Banks (formerly)
ASBMT	American Society of Blood and Marrow Transplantation
BLA	Biologics License Application
BMT	Bone Marrow Transplantation
CBB	Cord Blood Bank
CBU	Cord Blood Unit
CED	Coverage with Evidence Development
CIBMTR	Center for International Blood and Marrow Transplantation Research
CLIA	Clinical Laboratory Improvement Act
CMS	Centers for Medicare & Medicaid Services
CWBYCT	C.W. Bill Young Cell Transplantation
FACA	Federal Advisory Committee Act
FACT	Foundation for the Accreditation of Cellular Therapy
FDA	Food and Drug Administration
FY	Fiscal Year
GAO	Government Accounting Office
HHS	U.S. Department of Health and Human Services
HRSA	Health Resources and Services Administration
HSCT	Hematopoietic Stem Cell Transplantation
HCT/P	Human Cells, Tissues, and Cellular and Tissue-Based Products
IND	Investigational New Drug
IOM	Institute of Medicine
LDT	Laboratory-Developed Testing
MDS	Myelodysplastic Syndrome
MOU	Memorandum of Understanding
NCBI	National Cord Blood Inventory
NHLBI	National Heart, Lung, and Blood Institute
NMDP	National Marrow Donor Program
P.L.	Public Law

SCD	Sickle Cell Disease
TNC	Total Nucleated Cell

I. Legislative Language

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 a requirement in Section 3 that:

> *"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."*

II. 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 (CWBYCT) Program as the successor to the National Bone Marrow Donor Registry. The CWBYCT 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 CWBYCT Program also provides the infrastructure for data collection 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, as well as authorized federal support for the collection of high-quality, genetically diverse cord blood units. As the authorization process 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 an additional $10 million in funds for the National Cord Blood Stem Cell Bank Program. The Conference Report on "Cord Blood: Establishing a National Hematopoietic Stem Cell Bank Program," published in the *Congressional Record,* 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 IOM recommended a program structure similar to what was subsequently authorized by P.L. 109-129.

Slight differences exist between the program activities that were required by the National Bone Marrow Donor Registry and the current scope of the CWBYCT Program. Under current law, the CWBYCT Program may be administered through four infrastructure contracts rather than through a single contract, as was the case under the National Bone Marrow Donor Registry. 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. Additionally, P.L. 109-129 established a goal for the National Cord Blood Inventory (NCBI) Program 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 CWBYCT Program. Finally, the statute requires establishment of an Advisory Council to provide recommendations to the Secretary on activities related to these programs.

III. C.W. Bill Young Cell Transplantation Program Overview

As the successor to the National Bone Marrow Donor Registry, the CWBYCT 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 CWBYCT Program also enables data collection on the clinical outcomes of transplants. The CWBYCT Program is operated through four major contracts that were awarded through a competitive process in September 2012. Each contract has a 1-year base period and four possible 1-year options.

During FY 2014, the CWBYCT Program exercised the second option year of the four infrastructure contracts. (Figure 1 illustrates how both the CWBYCT Program and the NCBI Program were structured in FY 2014). The purpose of each CWBYCT Program contract is described below:

1. The Bone Marrow Coordinating Center contractor 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 contractor 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 contractor maintains 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 contractor also provides supportive services to patients in need of blood stem cell transplants from time of diagnosis through survivorship.

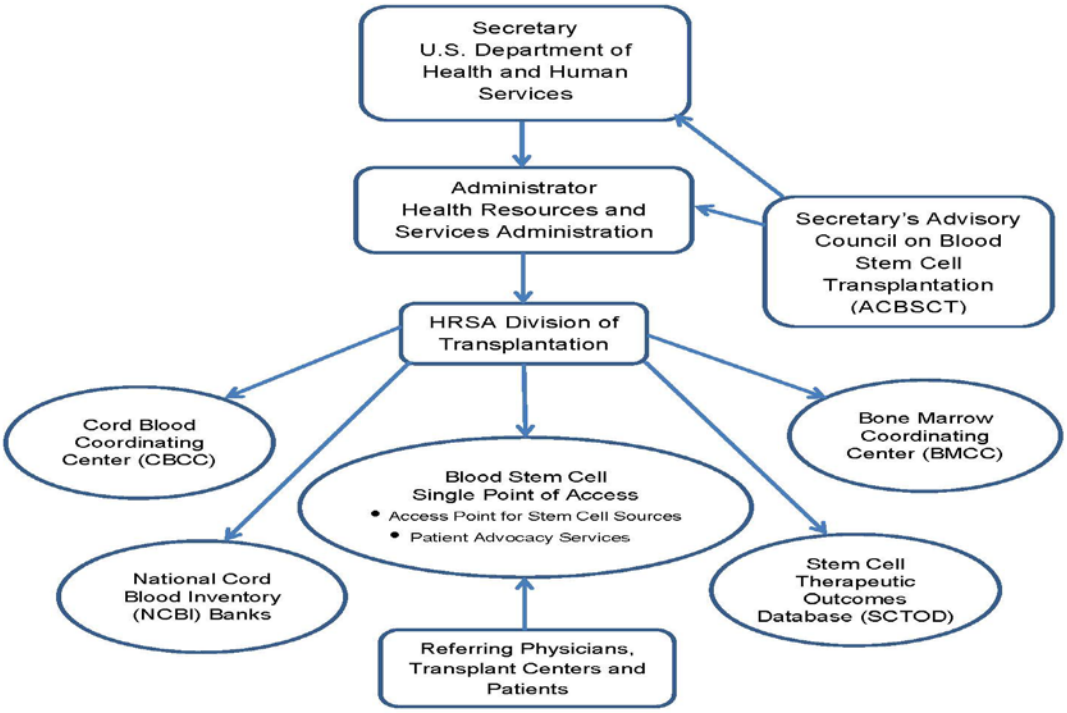
4. The contractor for the Stem Cell Therapeutic Outcomes Database (Outcomes Database) collects and analyzes data on the clinical outcomes of blood stem cell transplants. The Outcomes Database contractor is also charged with developing an approach to collect data on emerging therapeutic uses of cells found in umbilical cord blood, bone marrow, and peripheral blood (i.e., uses other than for hematopoietic reconstitution).

[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.

In FY 2014, the National Marrow Donor Program (NMDP) operated the Bone Marrow Coordinating Center, the Cord Blood Coordinating Center, and the Single Point of Access/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 contract to operate the Outcomes Database was held by the Center for International Blood and Marrow Transplant Research (CIBMTR) at the Medical College of Wisconsin. 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 CWBYCT Program contracts through FY 2014 can be found in Table 1.

Figure 1: C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory Program



HRSA's Healthcare Systems Bureau, Division of Transplantation is the operating division responsible for providing oversight of the CWBYCT and NCBI Programs. The CWBYCT 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 (NCBI) Banks" represents the 13 public umbilical cord blood banks under contract to HRSA for the NCBI Program. 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 of HRSA on matters carried out by both the CWBYCT and the NCBI Programs.

Table 1: Allocations for the C.W. Bill Young Cell Transplantation Program Contracts, FY 2006 – 2014

Fiscal Year	Appropriation +	Bone Marrow Coordinating Center*	Cord Blood Coordinating Center*	Single Point of Access/ 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
2013	$21,877,000	$13,948,000	$1,474,639	$723,623	$3,872,054	$20,018,316
2014	$22,054,000	$13,600,370	$1,495,000	$742,285	$3,959,158	$19,796, 813
Total	$211,499,000	$144,483,643	$9,461,902	$6,079,178	$33,340,831	$193,365,554

+Administrative costs account for the difference between the level of appropriations and the total funds awarded.
*These are cost-sharing contracts. The amount provided reflects the government's share.

A. Professional and Public Education Activities

In FY 2014, the contractors for the CWBYCT Program (i.e., the NMDP and the CIBMTR) continued to deliver educational resources and services to the physicians who refer patients for blood stem cell transplantation, those who treat patients with transplantation, and the general public. The NMDP carried out several initiatives to inform health professionals about the importance of timely transplant, when indicated, to improve patient outcomes. The NMDP also continued its Continuing Medical Education programs to raise 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 Stem Cell Transplantation (HSCT) because of lack of Medicare reimbursement for the procedure. In recent years, the number of patients receiving allogeneic HSCT for MDS has increased as a result of a research study being conducted by CIBMTR and approved by the Centers for Medicare & Medicaid Services (CMS) under Coverage with Evidence Development (see Table 2 below for number of patients by FY). This study will provide CMS with evidence regarding the effectiveness of HSCT in treating MDS to guide future policy decisions. At the end of FY 2014, 121 U.S. transplant centers had enrolled patients in this study.

Table 2: Number of patients 65 years of age or older registered with the Center for International Blood and Marrow Transplant Research that received an allogeneic transplant at a U.S. transplant[2] center for MDS in FYs 2010-2014

Fiscal Year	Related Donor	Unrelated Donor	Total
2010	44	61	105
2011	43	91	134
2012	68	146	214
2013	89	207	296
2014	111	198	309

Other professional education and outreach efforts included (1) hosting a stakeholder forum to address financial barriers to transplantation; (2) working with medical schools to encourage a clinical rotation in the area of bone marrow transplantation (BMT); and (3) continuing public outreach and education efforts, particularly on college campuses, to add more volunteer donors to the registry.

Public and professional engagement regarding value in stem cell transplantation

In FY 2014, the NMDP hosted a 2-day working forum entitled "Defining Quality and Value in Stem Cell Transplantation" to bring stakeholders together to discuss ways to account for cost and quality when measuring the value of blood stem cell transplantation. The forum consisted of over 100 stakeholders from the stem cell transplantation community, including patient advocacy organizations; medical and program directors from transplant centers; payers; and leadership from the American Society of Blood and Marrow Transplantation (ASBMT), the Foundation for the Accreditation of Cellular Therapy (FACT), and the CIBMTR.

Nationally recognized speakers discussed their perspectives on how the blood stem cell transplantation field could develop approaches to measure quality and improve value for patients. Many approaches centered on coordination of care, public accountability, process improvement, and the overall patient experience. Meeting participants made a number of recommendations regarding timely referral to transplant, recognizing that early referral could greatly impact overall care experience, and that payer involvement with referral timing was crucial to success. Other recommendations addressed transplant center data and the need to investigate further how the blood stem cell community could leverage data from CIBMTR and other sources to identify utilization patterns and improve transplant outcomes.

[2] In previous reports to Congress, this table reported only first allogeneic transplants, which understates the number of transplants performed. It was modified to include all allogeneic transplants performed at U.S. transplant centers for MDS.

Professional education and outreach through medical education: Bone Marrow Transplantation Curriculum Modules (English, Spanish)

Other professional education and outreach efforts in FY 2014 included the launch of 16 Core Curriculum education modules developed by the NMDP to encourage more physicians to choose the field of HSCT. A website was utilized to provide more medical students, residents, and fellows with an opportunity to gain exposure to the biology and clinical practice of HSCT. The NMDP and the ASBMT worked with medical schools to promote the availability of the modules on the NMDP's website at https://bethematchclinical.org/Resources-and-Education/Education-Courses-and-Events/Curriculum/. Data will be collected in 2015 to determine how often the site is accessed for educational outreach.

Public outreach and education on college campuses

In 2014, the CWBYCT Program continued its public outreach and education activities on college campuses with an emphasis on Historically Black Colleges and Universities, as well as Hispanic-Serving Institutions. The CWBYCT Program also continued to engage families of current patients searching for a match with the intent of increasing the number of volunteer donors listed on the adult donor registry. Family members are important advocates and partners in raising awareness about the unrelated donor registry. Additionally, the CWBYCT Program continued to research, support, and disseminate post-transplant guidelines and care plans that referring physicians can apply when their patients return to their care after undergoing transplantation.

B. Transplant Survival Rates

The CWBYCT Program had a long-term goal of increasing the 1-year survival rate of unrelated blood stem cell transplant patients from 62 percent in FY 2003 to 69 percent in FY 2010. To achieve this goal, the CWBYCT 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, enabling more patients to proceed to transplant at the clinically optimal time;
4. used data submitted to the Outcomes Database to refine transplant protocols and patient selection to better determine which patients will benefit from transplant;
5. developed tools to support transplant patients as they transition into long-term survivorship; and
6. grew the number of adult volunteer donors and cord blood units available through the CWBYCT Program to optimize the number of patients who are able to receive appropriately matched blood stem cells.

The 1-year survival rate increased from 62 percent in FY 2003 to 71 percent in FY 2010. The next goal addressing the 1-year survival rate was established for FY 2013, and the outcome data for the FY 2013 goal will not be available until FY 2015, as it takes 1 year to accrue the data post-transplant and 1 year to generate the report. This data will be reported in a future report.

IV. National Cord Blood Inventory Program 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 CWBYCT 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. The cost to recruit, collect, test, and cryopreserve cord blood units, and make them available for listing through the CWBYCT Program, varies by cord blood bank. In FY 2014, the range in costs for banks collecting for the NCBI Program was between $2,000 and $4,400 per unit.

HRSA awards contracts to public cord blood banks through the competitive Request for Proposal process. 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 the 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. Diversity of tissue types 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 Program 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 NCBI Program for 10 years from the date on which they last received funding from HRSA. 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.

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 CWBYCT 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 Program through FY 2014.

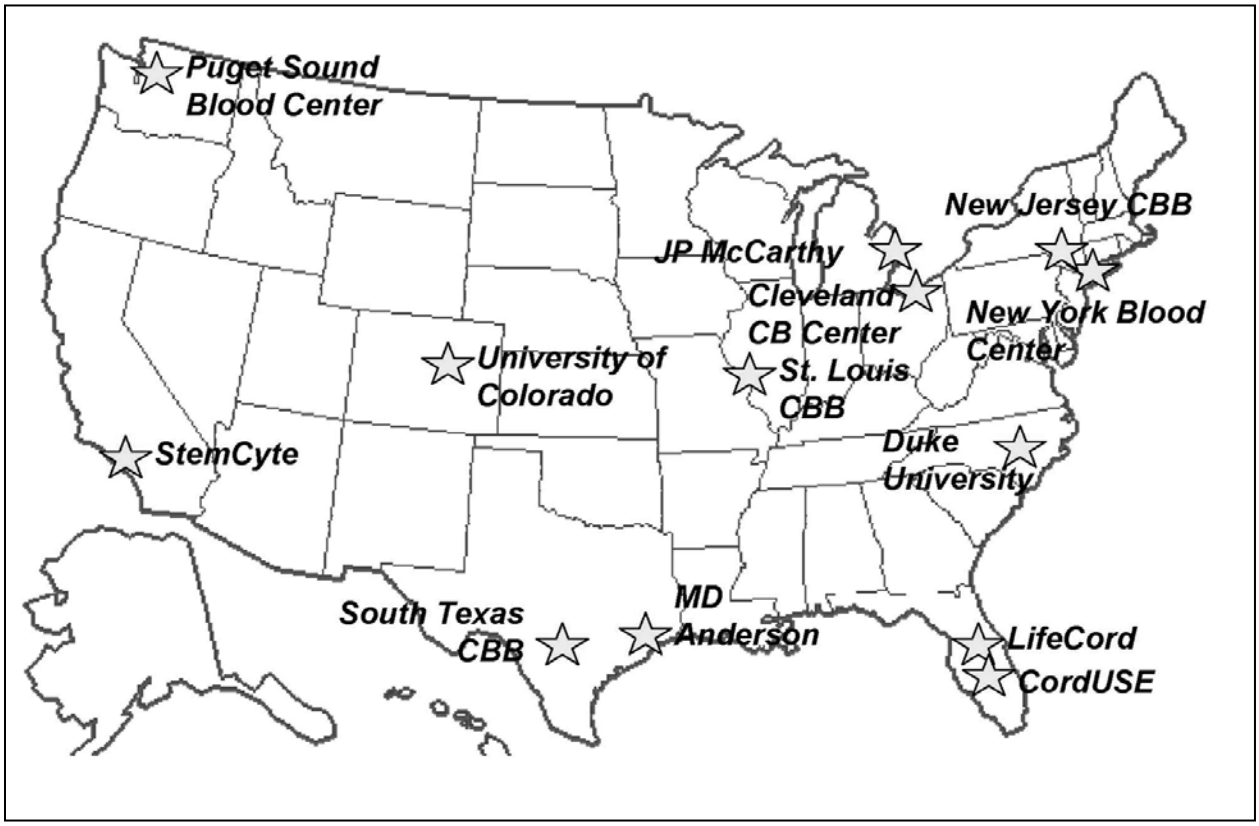
Table 3: Appropriations and Contract Allocation History for the National Cord Blood Inventory Program through FY 2014

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
2013	$11,147,000	$10,220,997	7,822
2014	$11,238,000	$10,398,185	7,329
Total	$106,711,000	$99,906,945	86,921

+Administrative costs account for the difference between the level of appropriations and total funds awarded.
*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 Program contracts since the inception of the NCBI Program through FY 2014 (five contractors hold two contracts each) and has provided funds to store nearly 80,000 NCBI units, 60 percent of which were from minority donors. Figure 2 identifies organizations holding an NCBI Program contract as of the end of FY 2014. The figure also shows the geographic distribution of NCBI Program contractors. 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 guarantee that diverse groups of donors are given the opportunity to donate cord blood.

Figure 2: National Cord Blood Inventory Banks



As of the end of FY 2014, 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), CORD: USE Cord Blood Bank (CORD: USE), 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).

A. Update on Changes Made to Provide Funding to National Cord Blood Inventory Cord Blood Banks

Since 2011, there have been changes in the way federal funds are distributed to NCBI contractors. These changes are a result of two reports which are (1) the HHS-issued *Interim Report on How Federal Funds are Distributed to Cord Blood Banks Participating in the National Cord Blood Inventory*; and (2) *Practices for Increasing Availability for Transplants and Related Challenges*, which was generated after the Government Accounting Office (GAO) conducted a study on the NCBI Program. This section provides a high-level overview of both reports, along with a description of some of the changes that have been implemented and ongoing assessments regarding the way federal funds are made available to the NCBI banks.

In August 2011, HRSA submitted an interim report to Congress that contained background information on both the CWBYCT Program and the NCBI Program. This report (1) described how federal funds were distributed to cord blood banks, (2) provided information on how cord blood banks contracted with cord blood collection sites, and (3) identified alternative ways federal funds could be distributed to cord blood banks. The report noted that the U.S. Department of Health and Human Services (HHS) would continue to work with the Advisory Council on Blood Stem Cell Transplantation (Advisory Council), the contractor for the Cord Blood Coordinating Center, NMDP, and the NCBI cord blood banks to identify and explore ways to: increase the number of public cord blood collection sites, increase the efficiency of cord blood collections, ensure progress toward a financially feasible and sustainable cord blood banking model, and improve upon existing NCBI Program contracting methods. The report also outlined options that would be considered for modifying the method for distributing funds to cord blood banks. Table 4 and the next section on the cord blood demonstration project for FY 2014 provide a summary of the options and changes in the way funds are provided to NCBI cord blood banks.

In October 2011, the GAO conducted a study of the NCBI Program to (1) identify practices to increase banking of cord blood units for the NCBI Program and related challenges, and (2) examine practices cord blood banks are using to lower costs and improve the efficiency of cord blood banking along with associated challenges. The GAO noted that the NCBI Program has increased the number of high quality, genetically-diverse units available for transplantation; and more patients, many with no other available graft source, were receiving access to cord blood units for transplantation. The report outlined challenges, including limited resources for cord blood banks to cover costs associated with hiring additional staff and increasing the number of cord blood units at existing and new collection sites.

Table 4: Options for Modifying the Method for Distributing Funds to Cord Blood Banks

Option	Status at end of FY 14	Changes Made	Next Steps
Provide, through the NCBI Program, a small amount of up-front funding to cord blood banks to initiate collections at new sites.	In FY 2014, a demonstration program was launched to provide up-front funding to cord blood banks to assist with costs related to initiating collections at new sites or expanding activity at existing collection sites.	An alternative approach has been identified to provide funding to NCBI banks, through the Cord Blood Coordinating Center, as part of a demonstration project.	The outcomes of the demonstration project will be assessed, and HRSA will make a determination regarding whether and how providing up-front cost to NCBI banks resulted in cord blood units being more rapidly added to the inventory.

Option	Status at end of FY 14	Changes Made	Next Steps
Re-examine the race/ethnicity-based payment differential for cord blood units.	The cord blood banks still have the ability to propose different prices based on race and ethnicity.	No changes have been made at this time. This approach has remained effective as a means of incentivizing the collection of cord blood units from minority donors and concentrating the use of federal funds on the collection of cord blood units that cord blood banks consider to be the most difficult and expensive to collect.	This issue will continue to be assessed by HHS and its Advisory Council.
Reduce emphasis on discounted prices negotiated with cord blood banks during contract negotiations.	Although the government continues to encourage discounted prices on cord blood units, cord blood banks have been able to propose different prices for reimbursement during the time of contract negotiation.	NCBI cord blood banks are provided with an opportunity to propose revised prices during contract extensions. It is recognized that such an approach may result in higher per unit reimbursement rates that would result in fewer overall cord blood units being added to the inventory each year.	This issue will continue to be assessed by HHS and its Advisory Council.

Option	Status at end of FY 14	Changes Made	Next Steps
Provide payment for cord blood units collected remotely at hospitals with which the cord blood bank does not have a written agreement.	HHS funded a pilot project testing this model with three NCBI cord blood banks.	No changes have been made at this time. Currently, reimbursement is permissible if a cord blood unit collected remotely meets the established NCBI Program criteria. It is recognized that such a collection model could be valuable for reaching small populations with unique diversity. However, many cord blood banks have concluded that such a model is not cost-effective.	None.
Raise the minimum total nucleated cell (TNC) threshold for payment or devise an adjustable payment model that provides escalating funds based upon the cellular content of the cord blood unit such that larger cord blood units are reimbursed at a higher rate.	The criteria for NCBI Program reimbursement has remained the same with the minimum criteria at $90x10^7$.	No changes have been made at this time. The Advisory Council and its workgroups have examined whether HHS should change its criteria for TNC; however, a formal recommendation has not yet been made. It remains permissible for cord blood banks to propose a higher cell count; however, an adjustable payment model has not been established as part of the broader NCBI Program.	This issue will continue to be assessed by HHS and its Advisory Council.

The interim HHS report noted that HHS plans to finalize specific recommendations relating to the NCBI Program pending the outcome of ongoing work of the Advisory Council and several of its workgroups. The Advisory Council has not yet provided recommendations to the Secretary regarding policy matters that would change the way NCBI banks are funded (e.g., reimbursement criteria for collecting cord blood units, scientific factors for defining a high-quality cord blood

unit, and changes to specifications regarding minimum total nucleated cell counts for NCBI cord blood units) but it will continue to assess its previous discussions on these important policy matters. Once HHS has further reviewed these issues and considered applicable recommendations from the Advisory Council, it will include such recommendations in subsequent reports to Congress.

B. Demonstration Project to Facilitate the Expansion of Cord Blood Collections at New or Existing Birthing Sites

In 2014, the CWBYCT Program, through its Cord Blood Coordinating Center contractor, worked with five accredited cord blood banks (CBB) to develop comprehensive plans to increase the number of cord blood collections at new or existing birthing sites in racially- and ethnically-diverse communities. These cord blood banks aimed to educate and obtain consent from expectant mothers for the collection and banking of their umbilical cord blood units. Data were collected from the selected CBBs (i.e., Lifecord, MD Anderson CBB, Cleveland Cord Blood Center, New Jersey CBB, and Carolinas CBB) and analyzed quarterly to access impact.

Results

As of September 30, 2014, there were a total of 10 new collection sites and a total of 466 minority Cord Blood Units (CBUs) with 269 CBUs (57.7 percent) recorded as African-American. These collection amounts were lower than the 780 minority CBUs and 587 African-American CBUs previously projected for FY 2014. However, the cord blood banks were making significant improvements in the number of CBUs collected during the fourth quarter. Prior to the start of the demonstration project, the cord blood banks indicated that there could be some delays in getting started, particularly for new collections sites that needed new agreements to be in place. Despite the delayed start and initial results, this demonstration project is worthwhile, and additional resources have been planned for FY 2015 (as a continuation of the demonstration project).

Next Steps

HRSA is aware of the challenges that the banks encountered during the initial phase of this project. Strategies are in place to assist the cord blood banks and the collection sites to overcome barriers in FY 2015.

C. Additional Demonstration Project to Explore Cord Blood Options for Transplant Centers with Difficult Donor Searches

A patient without a fully-matched related donor may need to rely on a fully-matched unrelated donor or a haploidentical relative for transplant. These latter stem cell sources are considered acceptable alternatives by some transplant center programs, and the selection is often driven by institutional preference. Despite the fact that most patients would have a potential product

suitable for transplant, given the different treatment options, some still languish in the search for a fully-matched adult donor and may miss the chance to get to transplant in an early stage of their disease.

In an effort to minimize delays in progressing toward transplant, the CWBYCT Program is proposing to identify patients with difficult searches (i.e., unlikely to have fully-matched donors) to determine the feasibility of providing transplant centers with cord blood selection information and advice. Transplant centers will be provided a search strategy if they do not have a fully-matched donor option, which will include cord blood recommendations and clinical expertise from an established cord transplant physician, if desired. Transplant centers will be assessed on how often they follow recommended cord advice, how often physicians with cord blood expertise are consulted, and transplant center satisfaction with the service.

Details on the implementation of the demonstration project will be provided in a future report to Congress.

V. C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory Program Statistics

- Transplants facilitated by the CWBYCT Program grew from 2,519 in FY 2004 to 6,253 in FY 2014 (an increase of 148.2 percent). Transplants for minority patients represent 15.8 percent of the total number of transplants facilitated by the CWBYCT Program in FY 2014. In FY 2014:
    - 1,225 (20 percent) of the transplants used marrow products,
    - 4,068 (65 percent) of the transplants used peripheral blood stem cells, and
    - 960 (15 percent) of the transplants used umbilical cord blood.

- Transplants facilitated by the CWBYCT Program for minority patients were 394 in FY 2004 and 990 in FY 2014 (an increase of 151.3 percent).
    - The number of transplants for minority patients decreased from 992 in FY 2013 to 990 in FY 2014 (a decrease of 0.2 percent).
        - Transplants for Black or African-American patients decreased from 306 in FY 2013 to 302 in FY 2014 (a decrease of 1.3 percent).
        - Transplants for Asian patients increased from 208 in FY 2013 to 239 in FY 2014 (an increase of 14.9 percent).
        - Transplants for Hispanic or Latino patients decreased from 415 in FY 2013 to 411 in FY 2014 (a decrease of 1.0 percent).
    - Transplants for minorities that utilized adult donors as the source of blood stem cells grew from 652 in FY 2013 to 695 in FY 2014 (an increase of 6.6 percent).

- Growth in the availability of umbilical cord blood units has expanded access to transplant, particularly for minority patients. Despite the decrease in the number of cord blood transplants in 2013, due to patients receiving other therapies (e.g., haploidentical transplants using blood stem cell from a related donor), these life-saving products remain viable for providing access to transplantation.

    - Approximately 31 percent of all cord blood transplants performed in FY 2014 were for minority patients. In contrast, only 13 percent of transplants utilizing blood stem cells from adult donors were for minority patients in FY 2014.

        - Minority cord blood transplants dropped from 340 in FY 2013 to 295 in FY 2014 (a decrease of 13.2 percent).
        - The number of Black or African-American patients who received a cord blood transplant in FY 2014 decreased 14.2 percent over FY 2013.
        - The number of Asian patients who received a cord blood transplant in FY 2014 increased 9.4 percent over FY 2013.
        - The number of Hispanic or Latino patients who received a cord blood transplant in FY 2014 decreased 14.6 percent over FY 2013.

    - A greater percent of minority patients who received a transplant in FY 2014 received cord blood as compared to all patients who received a transplant. This is because cord blood transplantation requires less stringent matching criteria than the criteria required in marrow transplantation. As a result, minority patients without matching marrow donors are able to proceed to transplant utilizing cord blood as a stem cell source.

        - Of the minority patients who received a transplant in FY 2014, over 30 percent received cord blood as their source of blood stem cells.
        - Among all patients who received a transplant in FY 2014, only 15.4 percent received cord blood as their source of blood stem cells.

- Approximately 12.4 million potential donors, 26 percent of whom are from minority populations, were included in the CWBYCT Program registry as of September 30, 2014.

    - In FY 2014, a total of 338,642 potential adult donors of bone marrow or peripheral blood stem cells between the ages of 18-44 were added to the registry.

        - 191,016 (56 percent) of these potential adult donors self-identified as belonging to an underrepresented racial or ethnic minority group.

- Over 209,000 cord blood units were available through the CWBYCT Program as of September 30, 2014. This includes cord blood units made available through the CWBYCT Program by domestic and international umbilical cord blood banks. A subset of these cord blood units was funded through the NCBI Program.[3]

    - As of September 30, 2014, 69,903 NCBI cord blood units were available to patients through the CWBYCT Program.[4]

        - This represents an increase of 5,943 (9.3 percent) cord blood units since the last Report to Congress.
        - 62 percent of all NCBI cord blood units made available to patients through the CWBYCT Program are from minority donors.
        - Approximately 17,000 more units await listing or will be collected with funds awarded to cord blood banks through the end of FY 2014.

    - In FY 2014, a total of 544 NCBI cord blood units were released for transplantation bringing the total number of units released for transplantation since 2007 to 3,708 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.
        - In FY 2012, 714 NCBI cord blood units were released for transplantation.
        - In FY 2013, 714 NCBI cord blood units were released for transplantation.
        - In FY 2014, 544 NCBI cord blood units were released for transplantation.

- As of September 30, 2014, more than 41,000 cord blood units not appropriate for clinical transplantation have been made available for research purposes.

- In FY 2014, 2,214 related donor-recipient research specimens were submitted to the repository maintained by the NMDP, the contractor for the Bone Marrow Coordinating Center and the Cord Blood Coordinating Center. This represents an increase of 638 (41 percent) over FY 2013. The NMDP also maintains an unrelated donor-recipient sample repository. In FY 2014, 10,305 specimens were submitted, which is a decrease of 754 (7 percent) from FY 2013.

[3] The total number of cord blood units available through the CWBYCT Program includes cord blood units funded through the NCBI Program as well as other non-NCBI cord blood units meeting quality and safety criteria for use in blood stem cell transplantation.
[4] The NCBI cord blood units are a subset of the total inventory of public cord blood units available through the CWBYCT Program.

- In FY 2014, the number of studies in progress by the CIBMTR, the contractor for the Outcomes Database, was 209. Eighty-one peer-reviewed publications on blood stem cell transplantation resulted from completion of studies in progress. The studies were published in several journals including: *Blood, Journal of Clinical Oncology, and Biology of Blood and Marrow Transplantation.* A list of CIBMTR's 2014 methodological publications are posted on http://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx?year=2014.

VI. Advisory Council on Blood Stem Cell Transplantation

In accordance with the Stem Cell Therapeutic and Research Act of 2005, P.L. 109-129, as amended by P.L. 111-264 (section 379(a)(1)), the Secretary has also established the Advisory Council on Blood Stem Cell Transplantation. The Advisory Council advises the Secretary and the HRSA Administrator on matters related to the CWBYCT Program and the NCBI Program. The first meeting of the Advisory Council was held in January 2008 and, by the end of FY 2014, there have been 14 subsequent meetings. Although the first meeting of the Advisory Council was held in January 2008, HRSA began the process of establishing the Advisory Council, according to the requirements of the Federal Advisory Committee Act (FACA), as soon as the authorizing statute was signed by the President on December 20, 2005. Due to the statutory requirements, time, and effort required for the establishment of an Advisory Committee under FACA, HRSA could not convene a meeting earlier than January 2008.

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
- Maximizing the potential uses of cord blood

By the end of FY 2014, 26 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 with 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 CWBYCT Program website (http://bloodcell.transplant.hrsa.gov). Table 5 provides a summary of the 26 recommendations submitted to the Secretary as of September 30, 2014.

Table 5: Advisory Council on Blood Stem Cell Transplantation Recommendations

Meeting Date	Summary of Recommendation	Status of Recommendation
April 29, 2008	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.	FDA issued its guidance for industry on "Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications" on October 20, 2009, which addressed the Advisory Council's stated concerns. At the same time, 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 (IND) application to access unlicensed cord blood units when a suitable human leukocyte antigen 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. FDA has since revised that final guidance and finalized that draft IND guidance. The most current versions were issued in March 2014.
	2. The Advisory Council recommended that the Secretary restore full funding of the CIBMTR research focused cooperative agreement with the National Cancer Institute.	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.
December 16, 2008	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.	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 HSCT 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.

Meeting Date	Summary of Recommendation	Status of Recommendation
	4. The Advisory Council recommended that the CWBYCT Program implement policies that conform to a number of specific principles, which are included in the full recommendation, to protect the privacy and confidentiality of cord blood donors donating umbilical cord blood units to the CWBYCT Program.	In FY 2010, HRSA reviewed its contract specifications for the CWBYCT Program and the NCBI Program 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 FACT as accreditation organizations for the NCBI Program. 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 a Memoranda of Understanding (MOU) with two accrediting organizations. In FY 2012, HRSA signed an 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.

Meeting Date	Summary of Recommendation	Status of Recommendation
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 Blood 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.

Meeting Date	Summary of Recommendation	Status of Recommendation
	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.	In the licenses for the five HRSA-funded NCBI banks approved to date (starting November 2011); FDA has permitted attachment of a tag disclosing expiration date and other information.

Meeting Date	Summary of Recommendation	Status of Recommendation
	13. The Advisory Council recommended that the Secretary work with 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.	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. 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. 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 under license, 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.

Meeting Date	Summary of Recommendation	Status of Recommendation
	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.	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. 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.
May 11, 2011	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.	In December 2011, 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, who is engaged in solely recovering cells or tissues for the registered establishment, may be exempt from requirements to register and list with FDA, but must comply with all other applicable requirements in 21 CFR Part 1271. 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.

Meeting Date	Summary of Recommendation	Status of Recommendation
	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 and Additional Requirements for Manufacturers of Human Cells, Tissues, and Cellular and Tissue-Based Products (HCT/Ps)" (see Section IX. H).[5] This guidance provided information about environmental and monitoring control for recovery of HCT/Ps.
November 8, 2011	17. The Advisory Council recommended to the Secretary that the FDA recognize and accept laboratory-developed testing (LDT) performed in Clinical Laboratory Improvement Act (CLIA)-certified, high complexity histocompatibility laboratories.	This issue has been discussed at the HHS-Interagency Workgroup on Hematopoietic Stem Cell Transplantation. FDA published a "Proposed LDT Regulatory Oversight Framework" on September 30, 2014.[6] The framework is available for review and public comment. FDA is specifically seeking feedback on LDTs used in CLIA-certified, high-complexity histocompatibility laboratories for transplantation when those LDTs are used in connection with organ, stem cell, and tissue transplantation to perform high resolution allele typing; for antibody screening and monitoring; or for the purpose of conducting real and "virtual" crossmatch tests.
	18. The Advisory Council recommended that FDA broaden the 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 HSCT is the appropriate approach to treatment.	In November 2011, 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.

[5] This can be found at http://www.fda.gov/downloads/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/Tissue/UCM285223.pdf.
[6] This can be found at http://www.fda.gov/MedicalDevices/ProductsandMedicalProcedures/InVitroDiagnostics/ucm407296.htm.

Meeting Date	Summary of Recommendation	Status of Recommendation
	19. The Advisory Council recommended that all cord blood products have the same IND and BLA clinical indications; this is scientifically and medically sound.	The titles of the two FDA guidance documents issued in March 2014 applicable to licensed products and IND products both describe the clinical indication by referring to products "intended for hematopoietic and immunologic reconstitution in patients with disorders affecting the hematopoietic system."
	20. The Advisory Council recommended implementation of a transition plan, initially stated as on or before October 20, 2011, to allow time for FDA to review recommendations 17 and 18 above and to allow for implementation of any changes by transplant centers that will be needed 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. FDA continues to work with other sponsors to address the availability of cord blood products.
	21. The Advisory Council recommended that cord blood units (CBUs) collected, through distribution of kits sent to motivated maternal donors or obstetrical units by an obstetric provider, which meet all NCBI Program/FDA qualifications, be eligible for listing on the NCBI Program and for FDA licensure.	In May 2011, FDA confirmed that public cord blood banks, including those that hold NCBI Program contracts with HRSA, may apply for a BLA that covers remote kit collections. If a biologics license is granted to NCBI contracted cord blood banks which included the remote kit collection procedure in their applications, cord blood units collected in this way may be listed and included in the NCBI Program. In May 2012, the first NCBI cord blood bank submitted an application that included remote kit collection procedures. FDA approved the collection model and those units are eligible for NCBI Program 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.

Meeting Date	Summary of Recommendation	Status of Recommendation
May 16, 2013	23. The Advisory Council recommended that the Secretary consider appropriate mechanisms to assure that the revised National Heart, Lung, and Blood Institute (NHLBI) publication entitled "Management and Therapy of Sickle Cell Disease" includes expert opinion about the curative option of hematopoietic cell transplantation for this disorder.	The Advisory Council's recommendation was shared with NHLBI by the National Institutes of Health's ex-officio Advisory Council member. Some changes were made to the final publication that was released in 2014.
May 16, 2013	24. The Advisory Council recommended that HRSA undertake educational/outreach efforts to the Sickle Cell Disease (SCD) patient and provider community to educate them about the progressive nature of SCD, increasing morbidity and mortality in early adulthood (ages 16-35 years), and the role of HSCT and its complications.	HRSA is engaging in public and professional outreach at national and regional SCD conferences. HRSA is working to ensure that the appropriate audiences, including primary care physicians and hematologists, are reached.
September 15, 2014	25. The Advisory Council recommended that the Secretary recognize hematopoietic transplantation for sickle cell disease as a covered benefit for all federal programs for which the Secretary has appropriate responsibility and oversight.	At the end of FY 2014, the recommendation was under internal review at HRSA.

Meeting Date	Summary of Recommendation	Status of Recommendation
September 15, 2014	26. The Advisory Council recommended that the Secretary direct HRSA and other HHS agencies to collaborate with CIBMTR to review research level data collection on allotransplants performed for SCD and consider appropriate reimbursement to optimize research data collection. This review should include SCD-specific data elements collected, the completeness of data collection, and mechanisms for reimbursement.	At the end of FY 2014, the recommendation was under internal review at HRSA.

Summary and Conclusions

The report reflects the status of the CWBYCT Program and the NCBI Program. 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. Future annual congressional reports will provide information on the progress of the CWBYCT Program and the NCBI Program.