



**U.S. Department of Health and Human Services
Health Resources and Services Administration**

REPORT TO CONGRESS

**Fiscal Year 2017 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) and P.L. 114-104, includes a requirement in section 3 which states, in part:

“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 the fiscal year (FY) 2017 annual report to Congress, which provides background information about the programs authorized by the law, describes the structure and operation of each program, and provides statistical information about the programs from October 1, 2016, through September 30, 2017. Unless otherwise stated, information presented in the report is from FY 2017. This is an update to the FY 2016 report, which included information through September 30, 2016.

Highlights of this report include activities related to the C.W. Bill Young Cell Transplantation Program (CWBYCTP), such as an expanded pool of 19.3 million volunteer adult marrow registrants, improved survival rates for unrelated-donor transplantation, and increased access to transplantation for underrepresented racial and ethnic populations.

The National Cord Blood Inventory (NCBI) Program contracts with cord blood banks to purchase cord blood units (CBUs) to help meet the statutory goal of building a public inventory of at least 150,000 new, high-quality, genetically diverse CBUs. NCBI funds support the collection of NCBI CBUs, which increases access to transplantation. The NCBI continues to grow in size and diversity with over 92,546 CBUs made available on the donor registry through CWBYCTP. Approximately 60 percent of the NCBI CBUs banked were from donors who identified as a race other than White. Increasing the diversity of NCBI offers improved chances of transplantation for individuals who lack a suitably matched relative and cannot find an adequately matched unrelated donor through the donor registry. In 2017, 494 (or 47 percent) of the 1,050 total CBUs released for transplantation through the CWBYCTP were NCBI CBUs.



Fiscal Year 2017 Annual Progress Report on the C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory Program

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Acronym List

ACBSCT	Advisory Council on Blood Stem Cell Transplantation
AGFBT	Advisory Group on Financial Barriers to Transplant
ASBMT	American Society of Blood and Marrow Transplantation
BMCC	Bone Marrow Coordinating Center
CBB	Cord Blood Bank
CBCC	Cord Blood Coordinating Center
CBU	Cord Blood Unit
CED	Coverage with Evidence Development
CIBMTR	Center for International Blood and Marrow Transplant Research
CMS	Centers for Medicare & Medicaid Services
CWBYCTP	C.W. Bill Young Cell Transplantation Program
FY	Fiscal Year
HRSA	Health Resources and Services Administration
HSCT	Hematopoietic Stem Cell Transplantation
MDS	Myelodysplastic Syndrome
NCBI	National Cord Blood Inventory
NMDP	National Marrow Donor Program
OPA	Office of Patient Advocacy
P.L.	Public Law
SCTOD	Stem Cell Therapeutic Outcomes Database
SPA	Single Point of Access
SPA-CC	Single Point of Access-Coordinating Center
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) and P.L. 114-104, includes a requirement in section 3 which states, in part:

“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 Reauthorization Act of 2015 authorizes the C.W. Bill Young Cell Transplantation Program (CWBYCTP), the National Cord Blood Inventory (NCBI), and the Advisory Council on Blood Stem Cell Transplantation (ACBSCT). The Health Resources and Services Administration (HRSA), Healthcare Systems Bureau, Division of Transplantation, is responsible for providing oversight of CWBYCTP and NCBI Programs (see Figure 1).

By statute, the purpose of CWBYCTP is to increase the number of bone marrow and cord blood transplants for recipients suitably matched to biologically unrelated donors. CWBYCTP collaborates with those in the blood stem cell transplantation field to address the need of unrelated blood stem cell transplants for individuals in the United States who have leukemia, lymphoma, sickle cell anemia, or other inherited metabolic or immune system disorders. For some individuals, using marrow or cord blood from an unrelated donor may be their best chance to live longer, healthier lives.

CWBYCTP supports the infrastructure for identifying, matching, and facilitating the distribution of bone marrow and cord blood suitably matched to candidates in need of a blood stem cell transplant. As required by statute, CWBYCTP provides patient and donor advocacy services, case management services, data collection on transplant outcomes, as well as public and professional educational activities.

The NCBI Program contracts with cord blood banks (CBBs) to meet the statutory goal of building a public inventory of at least 150,000 new, high quality, and genetically diverse cord blood units (CBUs). These CBUs are available for transplantation through the CWBYCTP.

The role of the ACBSCT is to advise, assist, consult with, and make recommendations to the Secretary of Health and Human Services and the Administrator of HRSA on matters carried out by both CWBYCTP and the NCBI Program. This report provides information about CWBYCTP, NCBI, and ACBSCT, including the organizational structures, important activities performed, and accomplishments during the past fiscal year (FY).

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

Through its Bone Marrow Coordinating Center (BMCC), Cord Blood Coordinating Center (CBCC), Single Point of Access (SPA), and Office of Patient Advocacy (OPA) functions, CWBYCTP provides a structure to facilitate blood stem cell transplantation with blood forming cells from unrelated donors for individuals with leukemia and other life-threatening blood disorders. Through its Stem Cell Therapeutic Outcomes Database (SCTOD) function, CWBYCTP also enables the collection of data on the clinical outcomes of those transplant recipients as well as data on blood stem cell products. CWBYCTP operates these five functions through four major contracts awarded through a competitive process during September 2012 (see Figure 1). Each contract has a 1-year base period and four possible 1-year option periods. During FY 2017, CWBYCTP exercised the fourth and final option year for each of the four infrastructure contracts. The funding history for the CWBYCTP contracts is in Tables 1 and 2. The following is a description of the four major contracts:

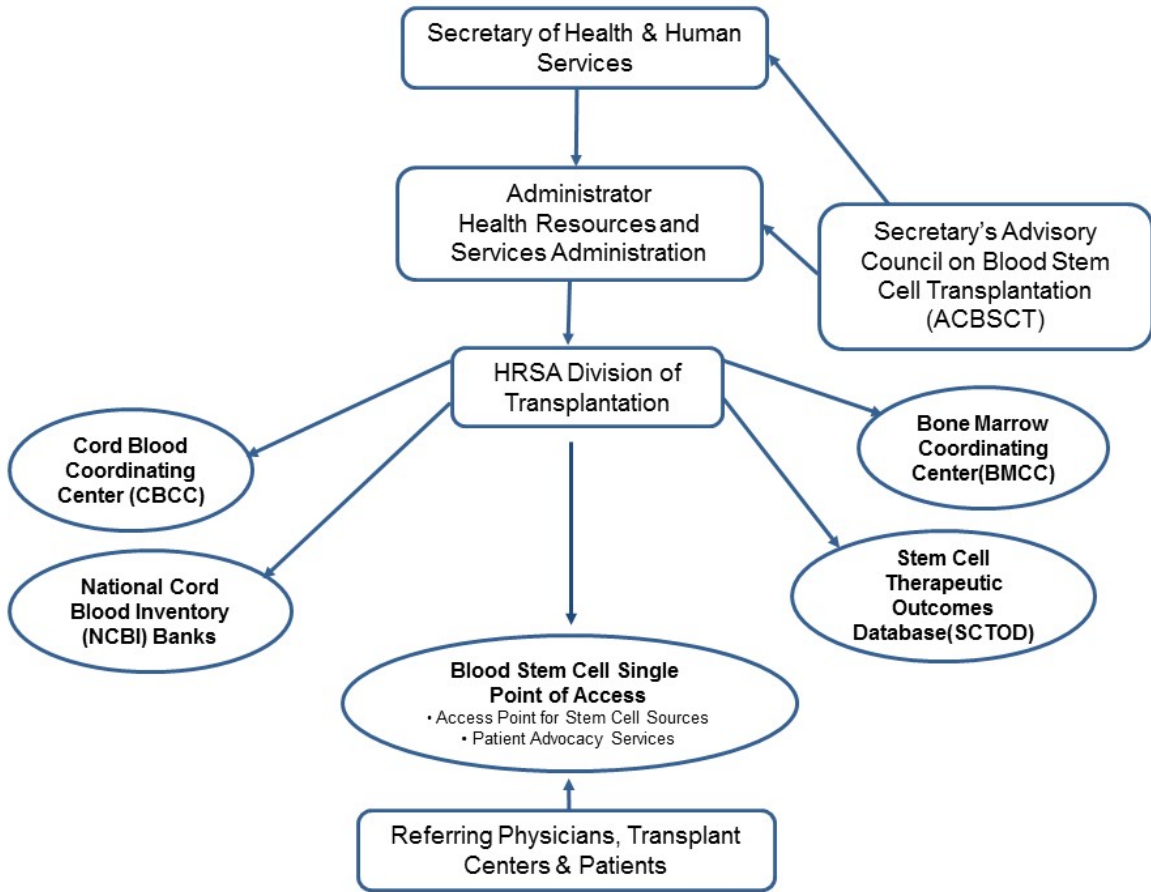
- The BMCC coordinates a network of organizations to recruit potential donors with an emphasis on the recruitment of individuals from diverse populations. This network collectively provides access to marrow transplants, provides tissue typing used to match recipients and donors, engages in public and professional educational activities related to blood stem cell donation, and works closely with other parts of CWBYCTP.
- The CBCC coordinates a network of public CBBs, engages in public and professional education activities related to umbilical cord blood donation and transplantation, and works closely with other parts of the CWBYCTP. CBBs support the network by building the NCBI through collecting and providing access to high-quality CBUs.
- The combined SPA and OPA maintain the single electronic system through which physicians can identify and access a suitable blood stem cell product from an adult donor or CBU, engage in public and professional educational activities related to treatment options, and provide supportive services to individuals in need of blood stem cell transplants from time of diagnosis through all stages during and after transplant.
- The SCTOD provides an electronic database of blood stem cell transplantation outcomes for use by researchers and health care professionals. The SCTOD also provides a repository that stores donor and patient samples for research and the collection and analysis of data on the clinical outcomes of blood stem cell transplant recipients and of data on blood stem cell products.

On September 30, 2017, the CWBYCTP used FY 2017 appropriations to develop and implement a new contracting structure (See Table 2. Table 1 shows the previous contracting structure). Based on a proactive assessment of the contracts, HRSA determined that a restructuring would be more beneficial to the government. For FY 2018, the SCTOD remains a separate contract.

OPA will operate as a separate contract continuing its efforts to engage in public and professional educational activities related to treatment options, and maintaining a system for patient advocacy. The BMCC, CBCC, and SPA functions are combined to form the Single Point of Access-Coordinating Center (SPA-CC) contract. The SPA-CC will maintain the electronic SPA system through which physicians, searching on behalf of patients, can search electronically for cells derived from adult marrow donors and CBUs and will support coordination activities for bone marrow and cord blood.

Since 2006, HRSA's contractor, the National Marrow Donor Program (NMDP) has held the competitively awarded contracts for the BMCC, CBCC, and SPA/OPA functions. NMDP is a not-for-profit organization based in Minneapolis, Minnesota, that manages the largest and most diverse bone marrow registry in the world and helps transplant patients without a matched relative to identify adequately matched volunteer adult marrow registrants or umbilical CBUs. Since 2006, the Center for International Blood and Marrow Transplant Research (CIBMTR) (known as the International Bone Marrow Transplant Registry from 1972-2004) at the Medical College of Wisconsin has held the competitively awarded contract for the SCTOD function. CIBMTR is the principal organization in the United States with the responsibility for collecting and analyzing data on the clinical outcomes of blood stem cell transplant recipients and of data on blood stem cell products. Funding for CIBMTR originates from financial partnerships with HRSA and the National Institutes of Health in the form of grants and contracts, and other financial support from non-federal entities.

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



Data Source: Figure created by HRSA and depicts contract structure through FY 17.

Table 1: Fund Allocations for the C.W. Bill Young Cell Transplantation Program Contracts, Fiscal Years 2007–2016⁺

FY	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
2007	\$25,168,000	\$21,125,660	\$0	\$0	\$2,240,629	\$23,366,289
2008	\$23,517,000	\$17,161,837	\$10,371	\$713,487	\$3,684,928	\$21,570,623
2009	\$23,517,000	\$16,691,921	\$717,570	\$659,928	\$3,663,174	\$21,732,593
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	\$22,109,000	\$13,600,370	\$1,495,000	\$742,285	\$3,959,158	\$19,796,813
2015	\$22,109,000	\$13,205,600	\$1,686,225	\$761,505	\$4,055,904	\$19,709,234
2016	\$22,109,000	\$12,415,360	\$1,937,198	\$781,302	\$4,156,111	\$19,289,971
Total	\$230,627,000	\$155,508,455	\$9,922,528	\$6,468,481	\$37,552,846	\$209,452,310

Data Source: Internal HRSA financial information.

+Administrative costs account for the difference between the level of appropriations and the total funds awarded.

Table 2: Fund Allocations for the C.W. Bill Young Cell Transplantation Program Contracts, Fiscal Year 2017 (for use in Fiscal Year 2018)

FY	Appropriation⁺	Single Point of Access-Coordinating Center[*]	Office of Patient Advocacy[*]	Stem Cell Therapeutic Outcomes Database	Total Obligation to Program Contracts
2017	\$22,109,000	\$14,640,000	\$802,849	\$4,305,380	\$19,748,229

Data Source: Internal HRSA financial information.

+Administrative costs account for the difference between the level of appropriations and the total funds awarded.

*On September 30, 2017, the CWBYCTP used FY 2017 appropriations to develop and implement a new contracting structure. The FY 2018 Report to Congress will discuss the new contracting structure in detail.

A. Professional and Public Education Activities

1. Educational Resources and Services

CWBYCTP delivered educational resources and services to physicians, other health care providers, and the public. These resources included online and in-person courses related to myelodysplastic syndromes (MDS), improving sickle cell disease outcomes, post-transplant care and screening recommendations, factors affecting quality of life after transplant, related donor transplant, and use of cord blood.

2. Centers for Medicare & Medicaid Services Coverage with Evidence Studies

To address concerns regarding lack of access to hematopoietic stem cell transplantation (HSCT) for some individuals diagnosed with MDS, the American Society of Blood and Marrow Transplantation (ASBMT), CIBMTR, NMDP, and other organizations requested a national coverage determination from the Centers for Medicare & Medicaid Services (CMS). This request resulted in a decision by CMS in 2010 to provide Coverage with Evidence Development (CED). Subsequently, CIBMTR developed two studies approved by CMS as fulfilling CED criteria. The largest study entitled, “Assessment of Allogeneic Hematopoietic Stem Cell Transplantation in Medicare Beneficiaries with Myelodysplastic Syndrome and Related Disorders” uses the SCTOD platform. Since approval in 2010, more than 2,300 individuals over age 65 received HSCT for MDS. Most of these individuals were Medicare beneficiaries (see Table 3 below for the number of individuals 65 years or older registered with CIBMTR by FY).

Table 3: Number of Individuals 65 Years of Age or Older Registered with the Center for International Blood and Marrow Transplantation Research who Received an Allogeneic Transplant at a U.S. Transplant Center for Myelodysplastic Syndrome in Fiscal Years 2010–2017⁺

Fiscal Year	Related Donor	Unrelated Donor	Total
2010	43	61	104
2011	43	91	134
2012	69	146	215
2013	89	209	298
2014	116	213	329
2015	122	277	399
2016	148	303	451
2017	147	239	386
Total	777	1,539	2,316

Data Source: CIBMTR.

+In previous reports to Congress, this table only reported the **first** allogeneic transplant for patients, which underrepresented the number of overall transplants performed. HRSA modified this table to include all allogeneic transplants, including those beyond the first allogeneic transplant, performed at U.S. transplant centers for MDS.

In 2016, CMS announced its decision to provide limited national coverage for allogeneic HSCT for individuals with sickle cell disease, multiple myeloma (allogeneic), and myelofibrosis within the context of CED. Prior to this CED, CMS excluded allogeneic transplants for multiple myeloma from coverage and was silent about coverage for sickle cell disease and myelofibrosis.

Current and pending research studies by CIBMTR addressing CED for the above indications include the following:

- The design for an additional CED study for sickle cell disease with broader eligibility criteria is under CMS review, activation is anticipated in 2018.
- AlloHSCT versus non-HSCT therapy for myelofibrosis (16 CMS-MF): CMS approval received in October 2016 and activated in November 2016.
- Allo versus autoHSCT for multiple myeloma (17-CMS-MM): CMS approval received in June 2017 and activated in July 2017.

3. Public and professional engagement regarding quality and value in stem cell transplantation

CWBYCTP engaged in activities through external committees, such as NMDP's Advisory Group on Financial Barriers to Transplant (AGFBT), which sponsored a forum on "Implementing Quality & Value in HCT" on October 19-20, 2016. This forum included topics suggested by the AGFBT, such as patient-centered care coordination, quality of life, value-based purchasing, early referrals, and underutilization of HSCT. The target audience of the forum was senior and executive-level leaders from the transplant community, physicians, program administrators, government agencies, national payers, purchasers, transplant networks, and stop-loss and reinsurer representatives. Working groups that helped plan the forum continue to meet on a regular basis to advance projects on these critical quality and value topics.

4. Professional education and outreach through medical education

Bone Marrow Transplantation Curriculum Modules

CWBYCTP offered 16 NMDP-developed educational modules designed to encourage more physicians to choose the field of HSCT. NMDP and ASBMT worked with medical schools to promote the availability of the modules on NMDP's website at <https://bethematchclinical.org/Resources-and-Education/Education-Courses-and-Events/Curriculum/> to educate medical students, residents, and fellows.

Education Program for Health Professionals

CWBYCTP offered multiple continuing education activities to inform health professionals of the latest developments in HSCT treatments, decision-making, and patient supportive care. CWBYCTP offered accredited continuing education courses for nurses, nurse practitioners, physicians, physician assistants, social workers, and other allied health professionals. These courses utilized multiple modes and formats to meet a range of educational needs, including 16 live webinars; 32 enduring/recorded programs; 7 symposia/meetings; 19 presentations (11 podium, 7 poster, 1 roundtable); and 17 exhibits at professional conferences and meetings. In

addition, CWBYCTP provided information about programs and research findings through publishing multiple manuscripts in peer-reviewed journals (access select list of publications at <https://bethematchclinical.org/research-and-news/browse-research>).

Updated mobile application for post-transplant guidelines

CWBYCTP updated the mobile application version of the post-transplant guidelines and care plans that referring physicians consult when their patients return to their care after undergoing transplantation. The Android link to the transplant guidelines is [https://play.google.com/store/apps/details?id=com.nmdp.webView&hl=en#\\$market://search](https://play.google.com/store/apps/details?id=com.nmdp.webView&hl=en#$market://search), and the iPhone link is <https://itunes.apple.com/us/app/transplant-hct-guidelines/id405310144?mt=8>.

B. CWBYCTP Statistical Updates

CWBYCTP serves a growing number of individuals in need of unrelated blood stem cell transplantation. Adding volunteer adult marrow registrants and high-quality diverse CBUs to CWBYCTP helps individuals without a matched family member explore viable options and identify potential matched blood stem cell sources.

- In 2017, the donor registry added 2,596,785 potential volunteer adult marrow registrants between the ages of 18-44 willing to donate bone marrow or peripheral blood stem cells to any patient. Of these newly added registrants, 195,191 (8 percent) self-identified as belonging to an underrepresented racial or ethnic population.
- By the end of FY 2017, CWBYCTP included approximately 19.3 million volunteer adult marrow registrants.
- More than 3.7 million of the registrants (approximately 19 percent) self-identified as belonging to an underrepresented racial or ethnic population.

It is important to increase the number of blood stem cell sources of umbilical cord blood and volunteer adult marrow registrants, particularly those from medically underrepresented racially and ethnically diverse populations, in order to increase access to blood stem cell transplantation. Increasing the number of blood stem cell sources of umbilical cord blood and volunteer adult marrow registrants addresses the statutory aim of ensuring that members of medically underrepresented racially and ethnically diverse populations, to the extent practical, have the same probability of finding a suitable unrelated donor as an individual who is not a member of an underrepresented population.

The number of unrelated blood stem cell transplants facilitated by CWBYCTP, including those for members of underrepresented racial and ethnic populations, decreased by 3 percent in FY 2017 over FY 2016 as indicated in Table 4. Preliminary information suggests, however, that overall patient access to potentially life-saving transplants did not decrease because transplant centers are getting better access to haplo-identical transplants from a half-matched family

member. Transplants of this type do not require facilitation through the CWBYCTP and may be responsible for some decreased demand for unrelated donors. Data from CIBMTR indicate that haplo-identical donor transplants are an increasing source of graft across races, with a slightly greater increase in distribution in Blacks or African Americans than Whites or other races, but data on long-term outcomes are not available. These observational data are only available through March 2017. HRSA will share updated information in a future report to Congress.

Table 4: Number of Transplants Facilitated by the C.W. Bill Young Cell Transplantation Program

Race/Ethnicity	FY 2016	FY 2017	Percent Change
American Indian or Alaska Native	27	24	-11%
Asian	216	205	-5%
Black or African American	301	225	-25%
Hispanic or Latino ¹	421	393	-7%
Multi-racial	28	22	-21%
Native Hawaiian or Other Pacific Islander	9	6	-33%
Unknown	1,337	1,427	7%
White	3,836	3,692	-4%
Total	6,175	5,994	-3%

Data Source: NMDP

Additionally, the CIBMTR portfolio included 222 studies in progress, which are studies that were not yet published. In 2017, 75 peer-reviewed publications involving blood stem cell transplantation resulted from completed studies. Several journals published the studies in progress, including the *Journal of the American Medical Association*, *Journal of Clinical Oncology*, *Leukemia*, *Blood*, *Cancer*, *American Journal of Hematology*, *Biology of Blood Marrow Transplantation*, *Clinical Pediatrics*, and *Bone Marrow Transplant*. A list of CIBMTR's 2017 publications is available at <https://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx?year=2017>.

C. Transplant Survival Rates

Because CWBYCTP supports individuals from diagnosis through transplant and throughout life after transplant, the program establishes goals not only for the number of transplants facilitated, but also for the outcomes of these transplants. As such, CWBYCTP activities include providing

¹ Hispanic or Latino may be any race.

a report on transplant center-specific survival rates. The transplant center-specific survival rate report provides potential HSCT recipients, their families and the public, with a comparison of survival rates among the centers in the CWBYCTP network. Transplant centers may also use the report as a basis for developing quality improvement initiatives. Extensive transplant and patient outcome data are available online at https://bloodcell.transplant.hrsa.gov/research/transplant_data/us_tx_data/index.html.

IV. National Cord Blood Inventory Program Overview

The NCBI Program contracts with CBBs to meet the statutory goal of building a public inventory of at least 150,000 new, high-quality, genetically diverse CBUs, which are made available to individuals, through CWBYCTP, on the donor registry. Donated CBUs that are not suitable for clinical transplantation are made available for research that is reviewed and approved by experts in the field of transplantation. The costs to recruit, collect, test, cryopreserve, and make CBUs available for listing through CWBYCTP varies by CBB. The average cost for banks listing CBUs for the NCBI Program through the CWBYCTP was \$1,365.68 per CBU.

HRSA awards contracts to public CBBs through a competitive process and reimburses CBBs on a per CBU basis for each CBU that meets all of the criteria specified in their contracts. The contracts specify the total number of reimbursed CBUs per year as well as the agreed upon racial/ethnic mix of donors (see more details in Table 6). Setting racial/ethnic collection goals helps to ensure that collected CBUs are from genetically diverse populations.

HRSA conducts annual reviews of each contractor's progress. The results of the reviews provide the basis for funding decisions. HRSA exercises options to support the banking of additional CBUs, subject to the availability of funds, for contractors who demonstrate the ability to meet CWBYCTP's goals as identified by the authorizing statute (including the ability to collect and store diverse, high-quality CBUs for unrelated donor transplantation). Funding decisions aim to ensure progress toward achieving the goal of banking at least 150,000 new CBUs while ensuring continued growth in the diversity of the available inventory. Table 5 shows the NCBI Program's appropriations and funding history as well as the number of CBUs contracted.

Table 5: Appropriations and Contract Allocation History for the National Cord Blood Inventory Program

Fiscal Year	Appropriation⁺	Total Contract Award Amount	Number of NCBI CBUs Contracted[#]
2004 – 2007*	\$27,719,522	\$27,691,700	23,049
2008	\$8,842,770	\$8,287,707	8,938
2009	\$11,983,000	\$11,240,309	10,207
2010	\$11,983,000	\$11,500,295	9,900
2011	\$11,910,037	\$11,384,224	10,571
2012	\$11,887,490	\$10,976,299	9,162
2013	\$11,265,712	\$10,220,997	7,900
2014	\$11,238,000	\$10,398,185	7,469
2015	\$11,266,000	\$10,404,320	6,469
2016	\$11,266,000	\$10,426,197	5,840
2017	\$12,266,000	\$11,329,136	6,369
Total	\$141,627,531	\$133,859,369	105,874

Data Source: Internal HRSA financial information.

+Administrative costs account for the difference between the level of appropriations and total funds awarded.

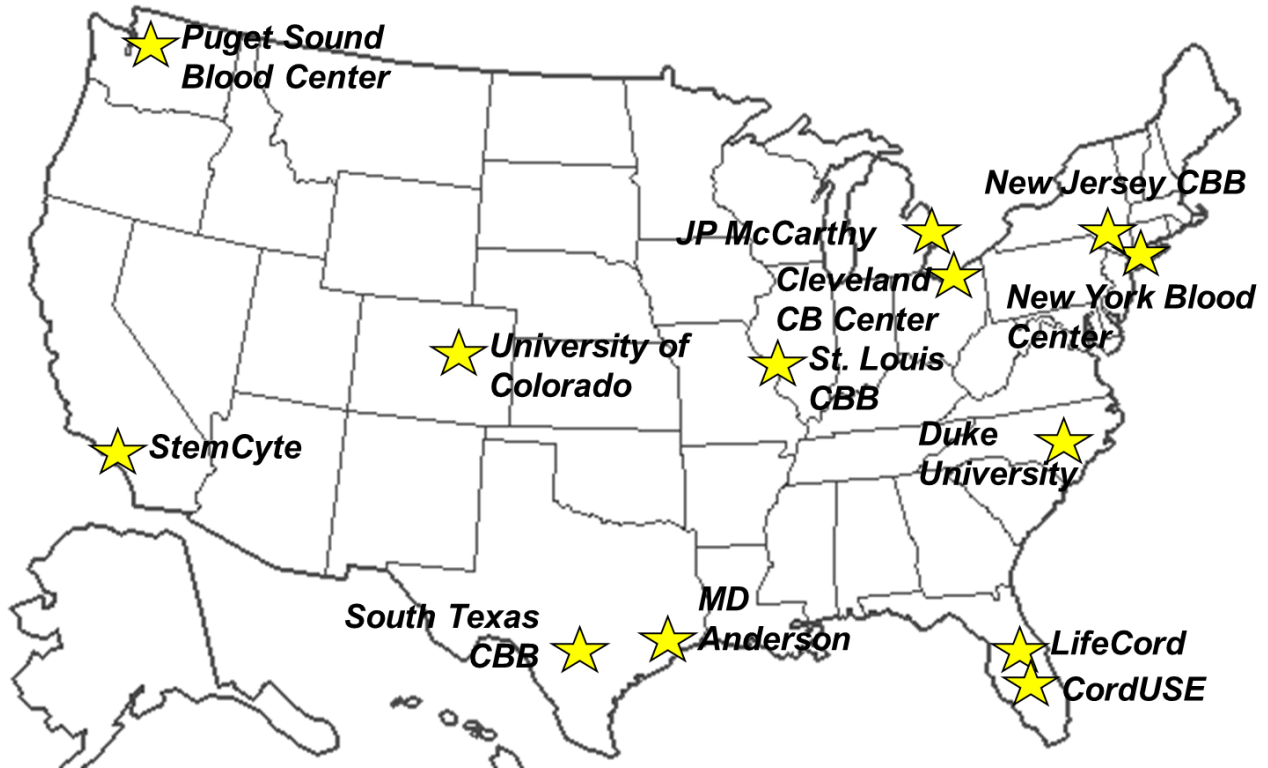
*Contract awards during FY 2007 used funds from no-year appropriations for FYs 2004-2006 and annual appropriations for FY 2007. Per the Appropriation Act for FY 2004, \$988,190 was awarded for a contract with the Institute of Medicine to recommend a structure for the National Cord Blood Stem Cell Bank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

#Modification to contracts may result in the number of CBUs funded by the NCBI (also known as NCBI CBUs) varying from prior year reports.

From the FY 2004 inception of the NCBI Program through FY 2017, HRSA awarded 18 NCBI Program contracts to 13 different contractors (5 contractors hold 2 contracts each), and the NCBI Program reimbursed 89,291 NCBI CBUs cumulatively,² approximately 60 percent of which were donations from underrepresented racial and ethnic populations. Figure 2 identifies organizations holding an NCBI Program contract as of the end of FY 2017. The figure also shows the geographic distribution of NCBI Program contractors. Geographic dispersion not only ensures continued availability of CBUs should a disaster temporarily impact one region of the country, but it also helps to guarantee that ethnically diverse CBUs will be donated and available to help more individuals in need.

² Actual number of units reimbursed by HRSA as of the end of FY 2017.

Figure 2: National Cord Blood Inventory Banks



Data Source: Figure created by HRSA with publicly available information. As of the end of FY 2017, HRSA contracted with 13 umbilical CBBs who collected, tested, stored, and distributed CBUs for the NCBI. Those contractors include 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. NCBI Program Accomplishments and Statistical Highlights

As of September 30, 2017, funds awarded from FY 2007 through FY 2017 (see Table 6) contracted for 105,874 CBUs. These CBUs represent approximately 71 percent of the statutory goal of at least 150,000 new, high quality, and genetically diverse CBUs. A total of 92,546 cord blood collections were made available through September 30, 2017 (see Table 7) and, since 2007, approximately 37 percent (5,341) of the 14,555 CBUs (NCBI and non-NCBI) released for transplantation through CWBYCTP used CBUs selected from the NCBI CBU inventory (see Table 8).

In addition to increasing the NCBI inventory, the support provided to NCBI-contracted banks through the purchase of CBUs played an important role in stimulating the collection and banking of additional CBUs (non-NCBI CBUs) and increasing the total number of CBUs available for donation. Additionally, NCBI banks have provided researchers more than 93,844 medically unsuitable CBUs for a wide variety of research endeavors.

Increasing the genetic diversity of NCBI increases the chance for transplantation to those individuals who lack a suitably matched relative and cannot find an adequately matched unrelated donor through CWBYCTP. Approximately 60 percent of the total number of NCBI CBUs made available on the donor registry through September 30, 2017, come from racially and ethnically underrepresented donor populations. See Table 6 for a breakdown of CBUs contracted by the NCBI program by race and ethnicity.

Table 6: Contracted National Cord Blood Inventory Cord Blood Units by Race and Ethnicity³

Fiscal Year^Δ	Asian	AI/AN⁺	Black or AA[*]	Hispanic or Latino	Multi-racial	NH/PI[#]	White	Totals
2007	1,551	35	4,363	6,996	2,079	57	7,968	23,049
2008	269	8	1,058	3,325	961	4	3,313	8,938
2009	285	8	876	2,683	1,372	10	4,973	10,207
2010	509	14	661	3,487	1,301	0	3,928	9,900
2011	370	1	2,004	2,661	1,483	12	4,040	10,571
2012	480	16	1,421	2,943	1,415	8	2,879	9,162
2013	333	11	833	2,330	1,378	12	3,003	7,900
2014	552	12	1,302	1,504	1,061	29	3,009	7,469
2015	558	1	1,105	1,849	1,256	9	1,691	6,469
2016	460	2	1,057	1,589	914	3	1,815	5,840
2017	259	0	1,164	1,650	985	0	2,311	6,369
Total	5,626	108	15,844	31,017	14,205	144	38,930	105,874
% of Total	5.31%	0.10%	14.96%	29.30%	13.42%	0.14%	36.77%	100%

Data Source: Internal HRSA information.

+American Indian or Alaska Native

*Black or African American

#Native Hawaiian or other Pacific Islander

Δ Changes in contract requirements may change numbers from prior years.

³ Hispanic or Latino may be any race.

Table 7: Cord Blood Collections

Fiscal Year	Cumulative CBU's Made Available⁺
2007	2,017
2008	11,870
2009	22,920
2010	34,744
2011	43,340
2012	53,609
2013	63,960
2014	74,650
2015	79,276
2016	85,443
2017	92,546

Data Source: Internal HRSA information.

⁺ Due to the lag between when CBU's are collected and when they have been fully tested and qualified for listing on the donor registry, not all of the CBU's collected with funds from a given FY will be available on the donor registry during that same FY.

Table 8: Cord Blood Units Released for Transplantation

Fiscal Year	National Cord Blood Inventory CBU's Released for Transplantation⁺	Total CBU's Released for Transplantation through the C.W. Bill Young Cell Transplantation Program^{*+}
2007	4	648
2008	104	1,148
2009	408	1,360
2010	530	1,555
2011	690	1,676
2012	714	1,637
2013	714	1,575
2014	545	1,359
2015	609	1,393
2016	529	1,154
2017	494	1,050
Total	5,341	14,555

Data Source: NMDP

* Includes NCBI and non-NCBI CBU's.

+Data in this report may change due to delayed data responses. Updated information will be included in the next C.W. Bill Young report to Congress.

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

CWBYCTP continued a demonstration project to develop comprehensive plans to increase the number of CBUs collected from members of underrepresented racial and ethnic groups, with a special emphasis on examining CBUs with a total nucleated cell (TNC) count greater than 1.5 billion at new or existing CWBYCTP birthing sites. CBUs with a higher TNC count are highly correlated with transplant success and more likely to be selected by transplant centers for patients.

Ten CBBs received funding in FY 2017. The ten CBBs included New York Blood Center, LifeCord CBB, MD Anderson CBB, Cleveland Cord Blood Center, St. Louis CBB, New Jersey CBB, Gencure, ITxM, Colorado CBB, and Carolinas CBB. Participating CBBs provided collected data for analysis quarterly.

The participating CBBs experienced multiple challenges, such as delays getting into new collection sites, increased use of delayed cord clamping, which can decrease the likelihood of a CBU qualifying for use, and labor and delivery staff turnover. As of September 30, 2017, 581 CBUs collected from underrepresented racial and ethnic populations were added to the NCBI from the 10 collection sites with 172 CBUs (30 percent) having a TNC count greater than 1.5 billion. Pending funding availability, HRSA may continue this demonstration project in FY 2018 and provide updated information regarding this demonstration project in the FY 2018 report to Congress.

C. FY 2017 Demonstration Program: Explore Cord Blood Unit Selection Options for Transplant Centers with Difficult Donor Searches

CWBYCTP sponsored activities focused on cord blood, such as CBU selection options and the study of the biology of cord blood. In an effort to minimize delay in time to transplant, CWBYCTP performed a demonstration project to identify individuals with difficult searches (i.e., unlikely to have a fully matched adult donor). The demonstration project was designed to determine the feasibility of providing transplant centers with cord blood selection information and advice from physicians experienced in cord blood transplantation. Through the demonstration project, interested transplant centers received a search strategy if they did not have a fully matched adult donor option and had limited experience with selecting possible CBUs. During FY 2017, nine transplant centers received cord blood consultations on behalf of 11 patients. Five of the 11 patients did undergo transplants, and all five of those transplants incorporated the CBUs recommended by the consulting physician.

Additionally, the blood stem cell community held a summit focusing on enhancing understanding of cord blood stem cell biology and engrafting capabilities. The summit resulted in a recommendation to increase publications regarding optimal transplantation practices for

patients with non-malignant diseases and metabolic disorders in order to advance the field of cord blood transplantation. In FY 2017, the publication *Biology of Blood and Marrow Transplantation* published an article titled, “Optimal Practices in Unrelated Donor Cord Blood Transplantation for Hematologic Malignancies” focusing on cord blood transplants for patients with malignant disease. There are plans to include continued support for such publications moving forward.

During FY 2018, HRSA will continue the 2017 demonstration projects: 1) to provide any transplant center interested in receiving additional advice and consultation regarding appropriate donor graft selection (adult donors or cord blood units), and 2) to evaluate information collected in FY 2017 to understand better the biology of cord blood cells. HRSA will provide additional information regarding the FY 2017 demonstration projects in the FY 2018 report to Congress.

D. Completion of FY 2016 Study on Financial Feasibility of the Public Cord Blood Banking Industry

In FY 2017, a study contracted in 2016 by the Office of the Assistant Secretary for Health with the RAND Corporation on the financial sustainability of the public cord blood banking industry was completed. The study has been completed and is now publicly available on the RAND website at https://www.rand.org/pubs/research_reports/RR1898.html. The main objectives of the study were to: 1) describe the existing cord blood banking system, 2) assess current trends and economic relationships from the perspective of key stakeholders, and 3) provide recommendations for ways to improve the system.

RAND reported the following key findings: 1) the NCBI Program has significantly increased the publicly available cord blood inventory while the proportion of stem cell transplants using cord blood has been declining; 2) future demand for cord blood is uncertain, and stagnating demand may force some public CBBs to close; 3) cord blood transplantation is not a panacea stem cell treatment, but it is an important source of blood stem cells for certain populations; and 4) the RAND research team’s calculations found that the value of having a public cord blood banking system far outweighs its operational costs.

RAND stated that the public cord blood banking system is worthy of continued investment as a public good. The study made recommendations within two categories: 1) NCBI Program Structure, and 2) Cord Blood Banking System Operations as a whole.

RAND recommendations for the NCBI Program Structure are: 1) increase the diversity of the national inventory by providing funding that encourages banks to add collection sites where more minority CBUs can be collected, increasing subsidies for minority CBUs, and increasing the minimum TNC count threshold, especially for non-minority CBUs; 2) standardize and consistently fund the NCBI contracts to the extent possible, as the uncertainty of contract funding and renewal makes it difficult for CBBs to plan for the future; and 3) prepare

contingency plans that would secure the fate of CBUs stored at banks should they go out of business if the market consolidates.

RAND recommendations for the Cord Blood Banking System Operations are: 1) CMS should consider changes to reimbursement for stem cell transplants so acquisition costs can be reimbursed separately, 2) federal funding could encourage knowledge sharing across banks and mentoring for centers and physicians with less cord blood experience, and 3) give banks the option to release smaller NCBI-subsidized CBUs to research endeavors could help advance scientists' understanding of cord blood and expand its use for transplantation or other uses in the future.

V. 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 and P.L. 114-104 (section 379(a)(1)), the Secretary of Health and Human Services established ACBSCT. ACBSCT advises the Secretary and the HRSA Administrator on matters related to CWBYCTP and the NCBI Program. ACBSCT held its first meeting in January 2008, and, as of the end of FY 2016, ACBSCT had held 17 additional meetings. The ACBSCT did not meet in FY 2017.

The ACBSCT has submitted a total of 31 recommendations to the Secretary of Health and Human Services. All ACBSCT meetings are open to the public and announced in the *Federal Register*. Each meeting provides attendees with the opportunity to make public comments. The charter for ACBSCT, its membership roster, agendas for upcoming meetings and meeting summaries are available on the CWBYCTP website at <http://bloodcell.transplant.hrsa.gov>. More information regarding recommendations by the ACBSCT is available at http://bloodcell.transplant.hrsa.gov/about/advisory_council/recommendations/index.html.

Summary

By increasing the size and diversity of the donor registry, CWBYCTP and NCBI have enabled thousands of individuals who lack a suitably matched relative to explore viable options and identify a matched blood stem source. In less than 10 years, the CWBYCTP listed over 92,546 CBUs on the donor registry. With over 19 million volunteer adult marrow registrants, CWBYCTP continues to increase access to blood stem cell transplantation. These programs continue to enhance the lives of thousands of men, women, and children who need potentially life-saving blood stem cell transplants.