



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

**REPORT TO CONGRESS**

**Fiscal Year 2018 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) 2018 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, 2017, through September 30, 2018. Unless otherwise stated, information presented in the report is from FY 2018. This is an update to the FY 2017 report, which included information through September 30, 2017.

This report addresses 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).

Highlights of this report include CWBYCTP’s new contractual structure, professional and public educational efforts, and programmatic accomplishments.

The 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 and diversify with 96,977 NCBI CBUs available on the donor registry through CWBYCTP. Approximately 63 percent of these CBUs were collected from donors who identified as a race other than White. Increasing the diversity of the NCBI offers improved chances of transplantation for individuals who lack a matched relative and cannot find a suitably matched unrelated adult donor through the donor registry. In FY 2018, 493 (or 52 percent) of the 949 total CBUs released for transplantation through the CWBYCTP were CBUs from the NCBI.

The ACBSCT did not meet in FY 2017 or FY 2018.



# Fiscal Year 2018 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
ASBMT	American Society of Blood and Marrow Transplantation
CBB	Cord Blood Banks
CBU	Cord Blood Unit
CED	Coverage with Evidence Development
CIBMTR	Center for International Blood and Marrow Transplant Research
CMML	Chronic Myelomonocytic Leukemia
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 Transplants
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-CC	Single Point of Access-Coordinating Center

## 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, provides oversight of CWBYCTP and NCBI Program (see Figure 1).

The purpose of CWBYCTP is to increase the number of bone marrow and cord blood transplants for recipients 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 life-threatening diseases such as leukemia, lymphoma, sickle cell anemia, or other metabolic or immune system disorders. For some individuals using bone marrow or cord blood from unrelated donors 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 from unrelated donors suitably matched to candidates in need of hematopoietic stem cell transplants (HSCT). As required by statute,<sup>1</sup> CWBYCTP offers 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 conducted by both the CWBYCTP and the NCBI Program.

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<sup>1</sup> The Stem Cell Therapeutic and Research Act of 2005, P.L. 109-129 (section 379(h) and section 379A), as amended by P.L. 111 264 of the Public Health Service Act) and P.L. 114-104.

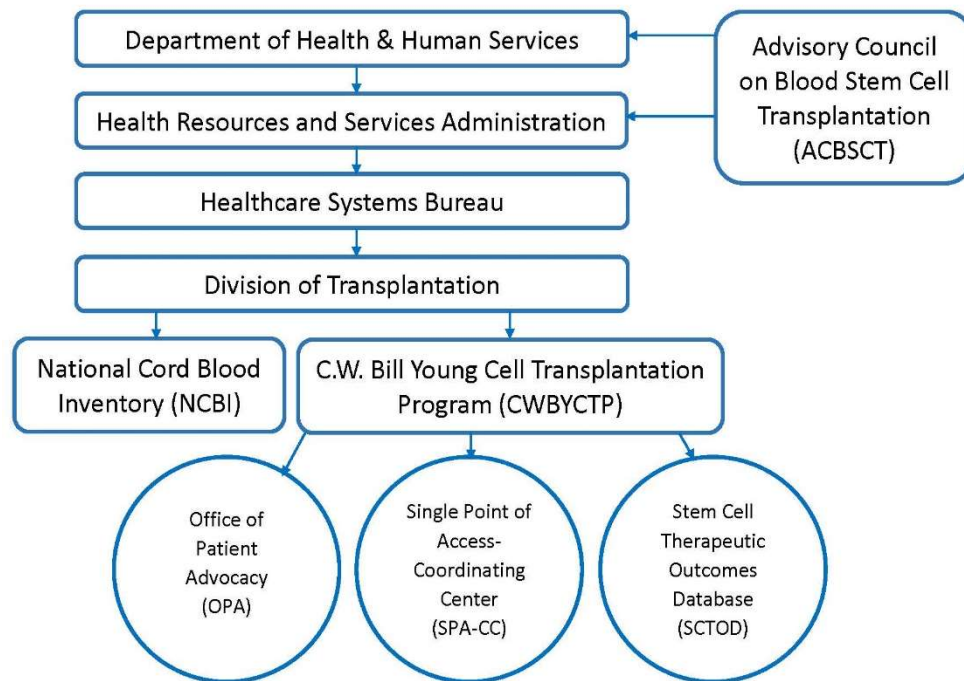
This report provides information about CWBYCTP, NCBI, and ACBSCT, including the organizational structures, important activities performed, and accomplishments during the past fiscal year (FY).

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

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, metabolic or immune system disorders. The CWBYCTP includes five functions (Bone Marrow Coordinating Center, Cord Blood Coordinating Center, Single Point of Access, Office of Patient Advocacy, and Stem Cell Therapeutic Outcomes Database) through three major contracts, which were awarded during a programmatic restructuring in September 2017 (see Table 2). For comparison, Table 1 shows award amounts of appropriated funds under the prior four-contract structure as included in earlier reports. Based on an assessment of the contracts, HRSA determined that restructuring the contracts from four contracts to three, by combining some of the functions, would be a more streamlined and beneficial approach for the government. The following is a description of the three current major contracts:

- The Single Point of Access-Coordinating Center (SPA-CC) contract includes a combination of the single point of access and bone marrow and cord blood coordinating centers' functions. The SPA-CC coordinates a network of organizations to recruit potential donors with an emphasis on the recruitment of individuals from diverse, underrepresented racial and ethnic populations. This network collectively provides access to bone marrow transplants, provides tissue typing to match patients and donors, and engages in public and professional educational activities related to blood stem cell donation. CBBs support the network by building the NCBI through collecting and providing access to high-quality CBUs. The SPA-CC maintains a single, searchable electronic system for health care professionals and physicians searching on behalf of patients for cells derived from adult bone marrow donors and CBUs through a single point of access.
- The Office of Patient Advocacy (OPA) contract supports patient advocacy and case management specific to bone marrow and blood stem cell transplantation, histocompatibility/search expertise, and guidance for patients and physicians. The OPA provides public and professional education, information, resources, and support for bone marrow transplant patients and families from diagnosis through survivorship.
- The Stem Cell Therapeutic Outcomes Database (SCTOD) contract supports an electronic database of blood stem cell transplantation outcomes for use by researchers and health care professionals. The SCTOD provides a repository that stores donor and patient samples for research and for the collection and analysis of data on clinical outcomes of HSCT recipients and of blood stem cell products.

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



**Data Source:** Figure created by HRSA that depicts contract structure in FY 2018.



**Table 1: Funding for the C.W. Bill Young Cell Transplantation Program Contracts Fiscal Years 2008–2016<sup>+</sup>**

FY	Appropriation	Contract Award Amounts				
		Bone Marrow Coordinating Center	Cord Blood Coordinating Center	Single Point of Access/Office of Patient Advocacy	Stem Cell Therapeutic Outcomes Database	Total, to Program Contracts
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

**Data Source:** Internal HRSA financial information.

+Secretary's Transfers and administrative costs account for the difference between the level of appropriations and the total funds awarded.

**Table 2: Funding for the C.W. Bill Young Cell Transplantation Program Contracts Fiscal Years 2017- 2018<sup>+</sup>**

FY	Appropriation	Contract Award Amounts			
		Single Point of Access-Coordinating Center*	Office of Patient Advocacy*	Stem Cell Therapeutic Outcomes Database	Total to Program Contracts
2017	\$22,109,000	\$14,640,000	\$802,849	\$4,305,380	\$19,748,229
2018	\$24,109,000	\$17,141,120	\$826,934	\$4,393,230	\$22,361,284

**Data Source:** Internal HRSA financial information.

+Secretary's Transfer and 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, more streamlined contracting structure that includes three contracts instead of four.

## A. Professional and Public Education Activities

### 1. Educational Resources and Services

CWBYCTP delivered over two dozen educational resources and services to physicians, other health care providers, and the public. These resources and services 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 HSCT for some individuals diagnosed with MDS, the American Society of Blood and Marrow Transplantation (ASBMT), Center for International Blood and Marrow Transplant Research (CIBMTR), National Marrow Donor Program (NMDP),<sup>2</sup> 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. From approval in 2010 through the end of FY 2018, more than 2,800 individuals 65 or older received HSCT for MDS. Most of these individuals were Medicare beneficiaries. See Table 3 below for the number of individuals aged 65 or older registered with CIBMTR by FY. Table 3 also includes chronic myelomonocytic leukemia (CMML) patients.

**Table 3: Number of Allogeneic Transplants for Myelodysplastic Syndrome in Patients ≥ 65 Years of Age Registered with the Center for International Blood and Marrow Transplant Research in Fiscal Year 2010 through Fiscal Year 2018, U.S. Centers Only (Chronic Myelomonocytic Leukemia included)**

<b>FY</b>	<b>Related Donor Transplants</b>	<b>Unrelated Donor Transplants</b>	<b>Total Transplants</b>	<b>Patients with more than 1 allogeneic transplant*</b>
2010	43	61	104	2
2011	43	90	133	6
2012	68	147	215	7
2013	89	209	298	4
2014	116	213	329	11
2015	121	278	399	18
2016	149	304	453	6
2017	175	292	467	8
2018	182	281	463	16
<b>Total</b>	<b>986</b>	<b>1,875</b>	<b>2,861</b>	<b>78</b>

**Data Source:** CIBMTR.

\*In some cases, the same patient received more than one transplant; this column identifies the number of patients who have received more than one allogeneic transplant for MDS and CMML since 2010.

<sup>2</sup> In FY 2018, CIBMTR was the contractor for the SCTOD contract, and NMDP was the contractor for the OPA and SPA-CC contracts.

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. Previously CMS excluded allogeneic transplants for multiple myeloma from coverage and was silent about coverage for sickle cell disease and myelofibrosis.

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

- Assessment of Allogeneic Hematopoietic Stem Cell Transplantation in Medicare Beneficiaries with Myelodysplastic Syndrome and Related disorders - Part I (10-CMSMDS): CMS approval received in December 2010 and activated in December 2010.
- A Study to Compare Allo-HSCT to Standard Care in Adolescents and Young Adults with Severe Sickle Cell Disease (Blood and Marrow Transplant Clinical Trials Network (BMT CTN)) #1503: CMS approval received in June 2016 and activated in November 2016.
- Prospective Assessment of AlloHSCT in Patients with Sickle Cell Disease (17-CMS-SCD): CMS approval received in October 2017 and activated in November 2017.
- Allo-HSCT versus non-HSCT therapy for myelofibrosis (16 CMS-MF): CMS approval received in October 2016 and activated in December 2016.
- Allo versus auto-HSCT 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 external activities such as the 2018 CIBMTR Center Outcomes Forum held on September 28, 2018. The Center Outcomes Forum is a bi-annual meeting to review and provide recommendations about the process, methodology, data collection and risk adjustment, and public reporting of the U.S. center-specific survival analysis. The CIBMTR invited representatives of the HSCT community, the ASBMT Quality Outcomes Committee, Foundation for the Accreditation of Cellular Therapy, NMDP, governmental funding agencies, patients, private payers, and statisticians to take part in discussions involving center-specific survival reporting, specifically to:

- Review recommendations for incorporating new variables in the center-specific analysis.
- Review recommendations about the proper use of statistical modeling.
- Discuss ways to enhance stakeholder collaborations aimed at affecting overall quality improvements of the data reporting

Some outcomes from the forum were:

- Enhanced collaboration between stakeholders to standardize processes and achieve quality improvements in reporting.
- Evaluated risk-adjustment modeling.
- Examined impact of public reporting of the U.S. center-specific survival analysis.

## 4. Professional Education and Outreach through Medical Education

### *Bone Marrow Transplantation Curriculum Modules<sup>3</sup>*

CWBYCTP offered 17 NMDP-developed educational modules designed to encourage more medical students and early career physicians to choose the field of HSCT. NMDP and ASBMT collaborated with medical school personnel to promote the availability of the modules on NMDP's website to educate medical students, residents, and fellows.

### *Transplant Referral Timing Guidelines and Resources*

CWBYCTP led the annual review and update of the Transplant Referral Timing Guidelines<sup>4</sup> through a comprehensive review and research process in collaboration with our partner ASBMT. The guidelines are produced in print, online, and mobile app versions; 2019 versions are currently available.

The NMDP and ASBMT developed and produced an Early Referral Slide Set<sup>5</sup> to address use of HSCT by cell source and number of transplants. This set also includes data on non-malignant and malignant diseases treated by transplant for use and reference by physicians and health professionals.

### *Education Program for Health Professionals*

CWBYCTP offered a variety of educational programs and resources along with continuing education activities to help health professionals provide the best care for patients before, during, and after transplant. CWBYCTP offered accredited continuing education activities as an approved provider for nurses through the American Nurses Credentialing Center, social workers through the Association of Social Work Boards, and laboratory professionals through the American Society for Clinical Laboratory Science. Continuing education activities included 4 conferences, 11 live webinars, and 20 on-demand activities, including a podcast. CWBYCTP offered a variety of non-accredited programs throughout the year, including exhibits at health professional conferences and meetings.

CWBYCTP also provided summaries<sup>6</sup> of HSCT-related research findings based on published manuscripts in peer-reviewed journals. The summaries and education activity updates were

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<sup>3</sup> More information about the Bone Marrow Transplantation Curriculum Modules is available at: <https://bethematchclinical.org/Resources-and-Education/Education-Courses-and-Events/Curriculum/>.

<sup>4</sup> More information about the Transplant Referral Timing Guidelines is available at: <https://bethematchclinical.org/transplant-indications-and-outcomes/referral-timing-guidelines/>.

<sup>5</sup> More information about the Early Referral Slide Set is available at: <https://www.asbmt.org/practice-resources/early-referral-slide-set>.

<sup>6</sup> Summaries are available at: <https://bethematchclinical.org/research-and-news/browse-research>.

shared through a subscription-based e-newsletter *Advances in Transplant* and distributed monthly to health professionals.

### *Mobile Application for Post-Transplant Guidelines*

CWBYCTP maintains 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. Continued review of published literature is undertaken and updates included on an annual basis. Both Android<sup>7</sup> and iPhone<sup>8</sup> links to the transplant guidelines are available.

## **B. CWBYCTP Statistical Updates**

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

- In FY 2018, the donor registry added 1,660,581 potential volunteer adult bone marrow registrants between the ages of 18-44 willing to donate bone marrow or peripheral blood stem cells to any patient.<sup>9</sup> Of these newly added registrants, 181,857 (11 percent) self-identified as belonging to an underrepresented racial or ethnic population.
- By the end of FY 2018, there were over 20.6 million volunteer adult bone marrow registrants. More than 3.8 million of the registrants (approximately 18 percent) self-identified as belonging to an underrepresented racial or ethnic population.<sup>10</sup>

Increasing the number of blood stem cell sources from umbilical cord blood and volunteer adult bone marrow registrants, particularly those from medically underrepresented racially and ethnically diverse populations, increases access to blood stem cell transplantation. Increasing the number of blood stem cell sources of umbilical cord blood and volunteer adult bone marrow registrants addresses the statutory aim of ensuring<sup>11</sup> that members of medically underrepresented racially and ethnically diverse populations 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 the CWBYCTP, including those for members of underrepresented racial and ethnic populations, increased by 2 percent in

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<sup>7</sup> The Android link is available at:

[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).

<sup>8</sup> The iPhone link is available at: <https://itunes.apple.com/us/app/transplant-hct-guidelines/id405310144?mt=8>.

<sup>9</sup> This number would appear to indicate a decrease in donor registration activity from FY 2017; however, the FY 2017 total included a one-time transfer of approximately 2 million internationally registered adult marrow donors so the increased FY 2017 were an anomaly.

<sup>10</sup> Total number of registrants vary from year to year due to new additions, file attrition and clean-up, and removal of donors no longer committed to staying on the registry.

<sup>11</sup> The Stem Cell Therapeutic and Research Act of 2005, Public Law (P.L.) 109-129 (section 379(e) and section 379(g)), as amended by P.L. 111 264 of the Public Health Service Act) and P.L. 114-104.

FY 2018 over FY 2017, as shown in Table 4. Table 4 also shows year over year gains in transplants for underrepresented racial and ethnic populations in most categories.

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

Race/Ethnicity	FY 2017	FY 2018	Percent Change
American Indian or Alaska Native	24	29	21%
Asian	205	248	21%
Black or African American	225	288	28%
Hispanic or Latino <sup>12</sup>	393	377	-4%
Multi-racial	22	22	0%
Native Hawaiian or Other Pacific Islander	6	10	67%
Unknown	1,427	1,361	-5%
White	3,692	3,780	2%
<b>Total</b>	<b>5,994</b>	<b>6,115</b>	<b>2%</b>

**Data Source:** NMDP finance department.

Additionally, the CIBMTR portfolio included more than 220 unpublished studies in progress. In FY 2018, completed studies resulted in 89 peer-reviewed publications<sup>13</sup> involving blood stem cell transplantation. Several journals published articles based on studies in progress including the *Journal of Clinical Oncology*, *Leukemia*, *Blood*, *Cancer*, *Biology of Blood Marrow Transplantation*, *Blood Advances*, and *Bone Marrow Transplant*.

## 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. CWBYCTP activities include providing 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

<sup>12</sup> Hispanic or Latino may be any race.

<sup>13</sup> The CIBMTR Publication List is available at <https://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx?year=2018>.

to develop quality improvement initiatives. Extensive transplant and patient outcome data are available online.<sup>14</sup>

## **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, available to individuals through CWBYCTP donor registry. Donated CBUs not suitable for clinical transplantation may be made available for research. 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 \$2,043.77 per CBU.<sup>15</sup>

HRSA awards contracts to public CBBs through a competitive process and reimburses CBBs on a per CBU basis for each CBU that meets all the criteria specified in the contracts. The contracts specify the total number of reimbursed CBUs per year and 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 by FY.

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<sup>14</sup> More information about transplant outcomes is available at:

[https://bloodcell.transplant.hrsa.gov/research/transplant\\_data/us\\_tx\\_data/index.html](https://bloodcell.transplant.hrsa.gov/research/transplant_data/us_tx_data/index.html).

<sup>15</sup> There are several variables used in calculating average CBU cost and any changes can impact the overall year-to-year average. Changes in the number of CBBs reporting, CBUs collected, total estimated banking costs, and cost per CBU collected will affect the average cost for banks listing CBUs for the NCBI Program through the CWBYCTP. There were 13 CBBs reporting in FY 2017 but only 12 CBBs reporting in 2018, and 20,938 fewer CBUs collected in FY 2018 (47,165) than in FY 2017 (68,103), all leading to an increase in the average cost per CBU in FY 2018.

**Table 5: Appropriations and Contract Funding History for the National Cord Blood Inventory Program<sup>16</sup>**

<b>Fiscal Year</b>	<b>Appropriation<sup>+</sup></b>	<b>Total Contract Award Amount</b>	<b>Number of NCBI CBUs Contracted<sup>#</sup></b>
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
2018	\$15,266,000	\$14,239,399	7,787
<b>Total</b>	<b>\$156,893,531</b>	<b>\$148,098,768</b>	<b>113,661</b>

**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.

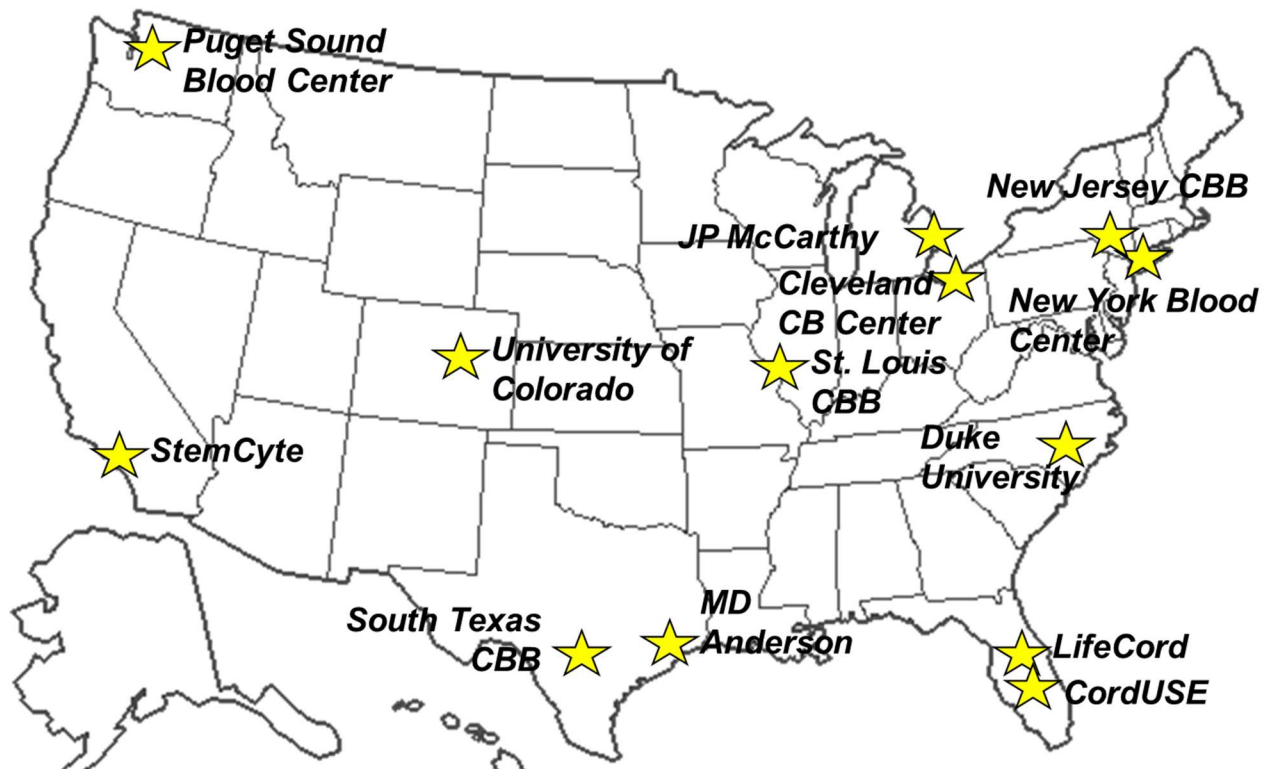
From the FY 2004 inception of the NCBI Program through FY 2018, HRSA awarded 18 NCBI Program contracts to 13 different contractors (5 contractors hold 2 contracts each), and the NCBI Program reimbursed 102,869 NCBI CBUs cumulatively,<sup>17</sup> approximately 63 percent of which were donations from underrepresented racial and ethnic populations. Figure 2 identifies organizations holding an NCBI Program contract as of the end of FY 2018. New York Blood Center completed its contract this year and sent it for close out in June 2018. 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.

<sup>16</sup> Table 5 and Table 6 show contracted NCBI CBUs in two different time spans: 14 years in Table 5 and 10 years in Table 6. The total contracted NCBI CBUs reported in Table 6, is 90,612, which is 23,049 less than the overall total contracted NCBI CBUs of 113,661 reported in Table 5.

<sup>17</sup> Actual number of units reimbursed by HRSA as of the end of FY 2018.



**Figure 2: National Cord Blood Inventory Banks**



**Data Source:** Figure created by HRSA with publicly available information. As of the end of FY 2018, 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, 2018, funds awarded from FY 2008 through FY 2018 contracted for 90,612 CBUs (see Table 6). These CBUs represent 60 percent of the statutory goal of at least 150,000 new, high quality, and genetically diverse CBUs. A total of 96,977 CBUs was collected and made available through September 30, 2018 (see Table 7). Since 2008, approximately 39 percent (5,830) of the 14,856 CBUs (NCBI and non-NCBI) released for transplantation, through the 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 furthering the collection and banking of additional CBUs (non-NCBI CBUs) and increasing the total CBUs available for donation. Additionally, since the inception of the NCBI, CBBs have provided researchers more than 94,000 medically unsuitable CBUs for a wide variety of research endeavors.

Increasing the genetic diversity of NCBI increases the chance of transplantation for those individuals who lack a suitably matched relative and cannot find a matched unrelated donor through CWBYCTP. See Table 6 for a breakdown of CBUs contracted by the NCBI program by race and ethnicity.

As shown in Table 8, the number of CBUs released for transplant has decreased since FY 2016, primarily due to an increased use of alternative therapies, including haploidentical transplants. Haploidentical transplants use blood stem cells from donors who are biologically related to the recipient-patients and are not facilitated through the CWBYCTP. Despite this recent decrease in CBU usage, patient access to potential life-saving treatments has not decreased. CBU collection and banking remain key in serving a diverse population. As the NCBI's inventory of CBUs grows and becomes more diverse, it will continue to provide increased access to a wider group of patients. CBUs from underrepresented racial and ethnic populations continue to account for over 50 percent of the CBUs contracted through the NCBI Program. HRSA will continue to monitor and assess trends in cord blood transplantation and share insights in future reports.

**Table 6: Contracted National Cord Blood Inventory Cord Blood Units by Race/Ethnicity<sup>18</sup>**

<b>Fiscal Year<sup>Δ</sup></b>	<b>Asian</b>	<b>AI/AN<sup>+</sup></b>	<b>Black or AA<sup>*</sup></b>	<b>Hispanic or Latino<sup>^</sup></b>	<b>Multi-racial</b>	<b>NH/PI<sup>#</sup></b>	<b>White</b>	<b>Totals</b>
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
2018	370	2	1,415	2,190	1,196	0	2,614	7,787
<b>Total</b>	<b>4,445</b>	<b>75</b>	<b>12,896</b>	<b>26,211</b>	<b>13,322</b>	<b>87</b>	<b>33,576</b>	<b>90,612</b>
<b>% of Total</b>	<b>4.91</b>	<b>0.08</b>	<b>14.23</b>	<b>28.93</b>	<b>14.70</b>	<b>0.10</b>	<b>37.05</b>	<b>100.00</b>

**Data Source:** Internal HRSA information.

<sup>Δ</sup> Changes in contract requirements may change numbers from prior years.

<sup>+</sup>American Indian or Alaska Native

<sup>\*</sup>Black or African American

<sup>^</sup>Hispanic or Latino may be any race.

<sup>#</sup>Native Hawaiian or other Pacific Islander

~ Data in this report may change due to delayed data responses and modification to contracts may result in the number of CBUs funded by the NCBI (also known as NCBI CBUs) to varying from prior year reports. The next report will include updated information.

<sup>18</sup> Table 5 and Table 6 show contracted NCBI CBUs in two different time spans: 14 years in Table 5 and 10 years in Table 6. The total contracted NCBI CBUs reported in Table 6, is 90,612, which is 23,049 less than the overall total contracted NCBI CBUs of 113,661 reported in Table 5. The NCBI was neither established nor began collecting NCBI CBUs until FY 2007; although, appropriations were received in FY 2004 through 2007.

**Table 7: Cord Blood Units Made Available for 2008-2018**

<b>Fiscal Year</b>	<b>FY Total of CBUs Made Available</b>	<b>Cumulative CBUs Made Available<sup>+</sup></b>
2008	9,853	11,870
2009	11,050	22,920
2010	11,824	34,744
2011	8,596	43,340
2012	10,269	53,609
2013	10,351	63,960
2014	10,690	74,650
2015	4,626	79,276
2016	6,167	85,443
2017	7,103	92,546
2018	4,431	96,977

**Data Source:** NMDP – Customer Ready Products

<sup>+</sup> Due to the lag between when CBUs are collected and when they have been fully tested and qualified for listing on the donor registry, not all of the CBUs 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 for 2008-2018**

<b>Fiscal Year</b>	<b>NCBI CBUs Released for Transplantation<sup>+</sup></b>	<b>Total CBUs Released for Transplantation through the CWBYCTP*</b>
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
2018	493	949
<b>Total</b>	<b>5,830</b>	<b>14,856</b>

**Data Source:** NMDP - Finance

\* Includes NCBI and non-NCBI CBUs.

<sup>+</sup> Data in this report may change due to delayed data responses. The next report will include updated information.

## **B. 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. To minimize delay in time to transplant, CWBYCTP initiated a demonstration project to identify individuals with difficult searches (i.e., unlikely to have a fully matched adult donor). The demonstration project, which was started in October 2017, was designed to determine the feasibility of providing transplant centers with cord blood selection information and advice from physicians experienced in cord blood selection and transplantation. Through the demonstration project, interested transplant centers, with limited experience in selecting suitably matched CBUs, received search strategy assistance if they did not have a fully matched adult donor option and could not identify a suitably matched CBU. During FY 2018, six transplant centers received cord blood consultations on behalf of seven patients. The search strategy assistance demonstration program resulted in four of the seven patients, whose donor searches were initially viewed as futile, receiving cord blood transplants.

Additionally, the blood stem cell community held a summit focusing on enhancing the understanding of cord blood stem cell biology and engrafting capabilities. The summit resulted in a recommendation to increase publications on the best transplantation practices for patients with non-malignant diseases and metabolic disorders to advance the field of cord blood transplantation. In FY 2018, as a result of this summit, 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 for similar publications in the future.

## **C. Support Cord Blood Banks with Existing Collection Efforts at Two Birthing Centers with High Birth Rates among Underrepresented Racial and Ethnic Populations**

In FY 2018, CWBYCTP approved a project for implementation in FY 2019, for the SPA-CC to provide support to NCBI contractors with existing agreements at Grady Hospital (Atlanta, Georgia) and the Memorial Hermann Hospital System (Houston, Texas) to increase collection of CBUs among underrepresented racial and ethnic populations. A plan and report on these efforts details the process for the provision of support annually to Duke University to add 160 CBUs from underrepresented racial and ethnic populations at Grady Hospital and 400 CBUs from the same populations at Memorial Hermann Hospital System. HRSA will provide any major findings resulting from the special project in a future report to Congress.

## **D. Plan to Identify Priority Areas for Special Projects – As Identified by the Cord Blood Community**

In FY 2018, CWBYCTP asked the cord blood community, via the NMDP's Cord Blood Advisory Group, about priority areas for potential future demonstration projects or special studies. The Advisory Group, which includes representatives from CBBs and laboratories, along with transplant physicians, researchers, and others from across the public cord blood community, identified and ranked 16 priority areas. One of its main priorities is a focus on increasing cord blood utilization; therefore, in FY 2019, HRSA will continue to support a special study begun in FY 2018 designed to serve transplant centers interested in receiving additional advice and consultation regarding appropriate donor graft selection (either adult donors or CBUs) and better understanding the biology of cord blood cells. HRSA will provide more information about this study along with any other new demonstration projects/special studies in the FY 2019 report to Congress.

The FY 2017 report to Congress presented the findings from a FY 2016 study conducted by the Office of the Assistant Secretary for Health with the RAND Corporation on the financial sustainability of the public cord blood banking industry.<sup>19</sup> As a follow-up to the RAND report, in FY 2018, HRSA approved a planned FY 2019 project, examining how the SPA-CC's contractor currently engages with the cord blood community, how often this engagement takes place, and recommendations for enhanced stakeholder input impacting the cord blood field. Future considerations will also include whether such recommendations should apply to all newly added CBUs available through the CWBYCTP or are specifically recommended for CBUs supported by the NCBI. If the latter, it is expected then the cord blood community would provide HRSA a detailed rationale for the distinction. HRSA will provide any major findings resulting from this project in a future report to Congress.

## **V. Advisory Council on Blood Stem Cell Transplantation**

Per 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) of the Public Health Service Act), the Secretary of Health and Human Services established the ACBSCT. The ACBSCT advises the Secretary and the HRSA Administrator on matters related to the CWBYCTP and the NCBI Program. The ACBSCT held its first meeting in January 2008, and, as of the end of FY 2016, ACBSCT held 17 other meetings. The ACBSCT did not meet in FY 2017 or FY 2018. Its next meeting is scheduled for September 2019.

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<sup>19</sup> A summary of these findings can be found here in the FY 2017 CWBYCTP Report to Congress: <https://bloodcell.transplant.hrsa.gov/fy17cwbillyoungreporttocongress.pdf>.

The ACBSCT has made a total of 31 recommendations<sup>20</sup> 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.<sup>21</sup>

## Summary

By increasing the size and diversity of the donor registry, the CWBYCTP and the NCBI Program have enabled thousands of transplant candidates who lack suitably matched relatives to explore viable options and identify matched blood stem sources. Over a span of 12 years, the CWBYCTP listed over 96,977 CBUs on the donor registry. With over 20.6 million volunteer adult bone 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.

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<sup>20</sup> The ACBSCT recommendations are available at [http://bloodcell.transplant.hrsa.gov/about/advisory\\_council/recommendations/index.html](http://bloodcell.transplant.hrsa.gov/about/advisory_council/recommendations/index.html).

<sup>21</sup> Information is available at <http://bloodcell.transplant.hrsa.gov>.