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

# REPORT TO CONGRESS

# Fiscal Year 2019 Annual Progress Report on the C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory Program

# Executive Summary

This is the fiscal year (FY) 2019 annual report to Congress that 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) programs and their activities from October 1, 2018, through September 30, 2019.

The report provides background information about each program, describes their structure and operation, and provides statistical information about them. Unless otherwise stated, the information presented in the report is from FY 2019. This is an update to the FY 2018 report, which included information through September 30, 2018.

The purpose of CWBYCTP is to increase the number of bone marrow and cord blood transplants for recipients matched to biologically unrelated donors. CWBYCTP supports the infrastructure for identifying, matching, and facilitating the distribution of bone marrow and cord blood from unrelated donors for individuals in need of hematopoietic stem cell transplants. By the end of FY 2019, there were over 22 million volunteer adult bone marrow registrants.

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 101,120 NCBI CBUs available on the donor registry through CWBYCTP.

The ACBSCT advises the Secretary of Health and Human Services and the Administrator of the Health Resources and Services Administration on matters related to the CWBYCTP and the NCBI Program. The ACBSCT held a virtual meeting on September 10, 2019. Based on current data and reduction in observed and confirmed Zika infection, ACBSCT recommends that the Department of Health and Human Services review the current Food and Drug Administration Recommendations for Reducing the Risk of Zika Virus Transmission by Blood and Blood Components as the guidance relates to utilization of cord blood.

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. These programs continue to increase access to blood stem cell transplantation and to enhance the lives of thousands of men, women, and children who need potentially life-saving blood stem cell transplants.

HRSA
Health Resources & Services Administration

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# Acronym List

| | |
|---|---|
| ACBSCT | Advisory Council on Blood Stem Cell Transplantation |
| ASTCT | American Society of Transplantation and Cellular Therapy |
| CBB | Cord Blood Banks |
| CBU | Cord Blood Unit |
| CIBMTR | Center for International Blood and Marrow Transplant Research |
| CMS | Centers for Medicare & Medicaid Services |
| CWBYCTP | C.W. Bill Young Cell Transplantation Program |
| FDA | Food and Drug Administration |
| FY | Fiscal Year |
| HHS | Department of Health and Human Services |
| 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 reauthorizes 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 needs of 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 of these individuals, a transplant 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 for individuals in need of hematopoietic stem cell transplants (HSCT). As required by statute,[1] 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 (HHS) and the Administrator of HRSA on matters conducted by both the CWBYCTP and the NCBI Program.

[1] 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.

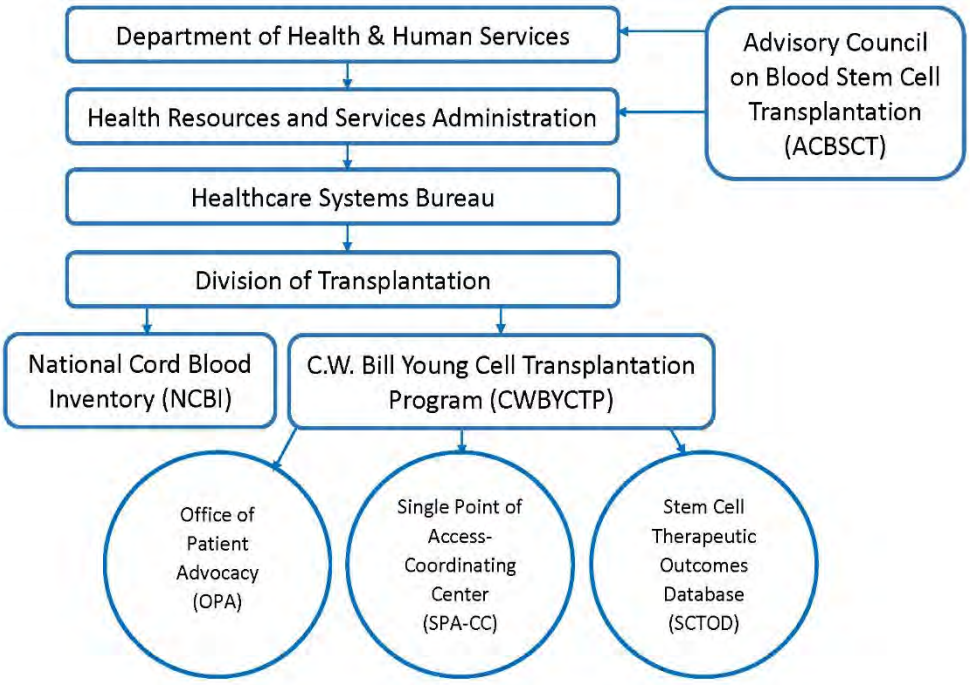
This report provides information about CWBYCTP, NCBI, and ACBSCT, including the organizational structures, activities performed, and accomplishments during fiscal year (FY) 2019.

# 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). 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. HRSA awarded the three major contracts during a programmatic restructuring in September 2017 (see Table 2). 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 legislative 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. The SPA-CC also contains a network of CBBs that lists its CBUs and makes them available for transplantation. 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:** Internal HRSA information.

**Table 1: Funding for the C.W. Bill Young Cell Transplantation Program Contracts Fiscal Years 2015–2016+**

| FY | Appropriations | Contract Awards | | | | |
|---|---|---|---|---|---|---|
| | | Bone Marrow Coordinating Center | Cord Blood Coordinating Center | Single Point of Access/Office of Patient Advocacy | Stem Cell Therapeutic Outcomes Database | Total Program Contracts |
| 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** | $44,218,000 | $25,620,960 | $3,623,423 | $1,542,807 | $8,212,015 | $38,999,205 |

**Data Source:** Internal HRSA financial information.
+Secretary's Transfers and administrative costs account for differences between appropriations and total program contracts awarded.

**Table 2: Funding for the C.W. Bill Young Cell Transplantation Program Contracts Fiscal Years 2017- 2019**[+]

| FY | Appropriations | Contract Awards* | | | |
|---|---|---|---|---|---|
| | | Single Point of Access-Coordinating Center | Office of Patient Advocacy | Stem Cell Therapeutic Outcomes Database | Total 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 |
| 2019 | $24,609,000 | $16,780,698 | $851,741 | $4,495,453 | $22,127,892 |
| **Total** | **$70,827,000** | **$48,561,818** | **$2,481,524** | **$13,194,063** | **$64,237,405** |

**Data Source:** Internal HRSA financial information.
+Secretary's Transfers and administrative costs account for differences between appropriations and total program contracts awarded.
*On September 30, 2017, the CWBYCTP used FY 2017 appropriations to develop and implement a more streamlined contracting structure that includes three contracts instead of four.

# Professional and Public Education Activities

## 1. Educational Resources and Services

CWBYCTP developed over three dozen educational resources and services for 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 the 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 Transplantation and Cellular Therapy (ASTCT - formerly known as the American Society of Blood and Marrow Transplantation), Center for International Blood and Marrow Transplant Research (CIBMTR), National Marrow Donor Program (NMDP),[2] 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. Subsequently, CIBMTR developed two studies approved by CMS as fulfilling these 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

[2] In FY 2019, CIBMTR was the contractor for the SCTOD contract, and NMDP was the contractor for the OPA and SPA-CC contracts.

in FY 2015 through the end of FY 2019, more than 2,300 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 in each FY.

**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 2015 through Fiscal Year 2019, U.S. Centers Only (Chronic Myelomonocytic Leukemia included)**

| FY | Related Donor Transplants | Unrelated Donor Transplants | Total Transplants | Patients with more than one allogeneic transplant* |
|---|---|---|---|---|
| 2015 | 121 | 278 | 399 | 17 |
| 2016 | 149 | 305 | 454 | 6 |
| 2017 | 178 | 296 | 474 | 7 |
| 2018 | 203 | 322 | 525 | 16 |
| 2019 | 166 | 350 | 516 | 8 |
| **Total** | 817 | 1,551 | 2,368 | 54 |

**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 Chronic Myelomonocytic Leukemia since 2015.

## 3. Professional Education and Outreach through Medical Education

*Bone Marrow Transplantation Curriculum Modules*[3]

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 ASTCT 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 through a comprehensive review and research process in collaboration with our partner, ASTCT. CWBYCTP produced the guidelines in print, online, and mobile app versions; 2019 versions are currently available online.[4]

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

[4] The 2019 Transplant Referral Timing Guidelines are available at: https://bethematchclinical.org/transplant-indications-and-outcomes/referral-timing-guidelines/.

The NMDP and ASTCT developed and produced an Early Referral Slide Set[5] to address the 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 3 conferences; 3 live webinars; and 28 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[6] of HSCT-related research findings based on published manuscripts in peer-reviewed journals. CWBYCTP shared the summaries and education activity updates through a subscription-based e-newsletter, *Advances in Transplant,* and distributed monthly to health professionals.

In addition to the published manuscripts, the CWBYCTP contractor's portfolio included more than 230 unpublished studies in progress. In FY 2019, the completed studies resulted in 95 peer-reviewed publications[7] involving blood stem cell transplantation. The following are seven of several journals that published articles based on completed studies:

1. *Journal of Clinical Oncology*,
2. *Leukemia*,
3. *Blood*,
4. *Cancer*,
5. *Biology of Blood Marrow Transplantation*,
6. *Blood Advances, and*
7. *Bone Marrow Transplantation.*

[5] More information about the Early Referral Slide Set is available at: https://www.astct.org/practice-resources/early-referral-slide-set.
[6] Summaries are available at: https://bethematchclinical.org/research-and-news/browse-research.
[7] The CIBMTR Publication List is available at https://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx.

FY 2019 completed studies:

- Published 23 patient-friendly summaries in FY 2019,[8] surpassing the SCTOD annual goal of 8 patient-friendly summaries. This helps fulfill the legislative requirement to make relevant scientific information not containing individually identifiable information available to the public in the form of summaries. A few of the topics covered include:
  - Comparative effectiveness in an older population[9] - Blood or marrow transplant helps treat leukemia after age 60;
  - Survivorship/late effects[10] - Survivorship care plans help transplant survivors;
  - Comparative effectiveness/graft source issues[11] - What happens when blood cells are used instead of bone marrow in BMT for children and teens with leukemia?;
  - Comparative effectiveness of preparative regimen alternatives[12] - Intravenous busulfan compared with total body irradiation pretransplant conditioning for adults with acute lymphoblastic leukemia.

*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[13] and iPhone[14] links to the transplant guidelines are available.

# 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 identify potential matched blood stem cell sources.

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[8] The CIBMTR Study Summaries for Patients is available at https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/pages/index.aspx.

[9] More information about comparative effectiveness in an older populations is available at: https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/Documents/2019.05.09-%20Ustun.pdf.

[10] More information about survivorship/late effects is available at: https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/_layouts/15/WopiFrame.aspx?sourcedoc=/ReferenceCenter/Patient/PatientSummaries/Documents/2018.12.04-Majhail.pdf&action=default.

[11] More information about comparative effectiveness/graft source issues is available at: https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/Documents/2018.08.21-Keesler.pdf.

[12] More information about comparative effectiveness of preparative regimen alternatives is available at: https://www.cibmtr.org/ReferenceCenter/PubList/Pages/PublicationDetail.aspx?pubid=a020L00000FFGJSQA5&title=Intravenous%20busulfan%20compared%20with%20total%20body%20irradiation%20pretransplant%20conditioning%20for%20adults%20with%20acute%20lymphoblastic%20leukemia.

[13] The Android link is available at: https://play.google.com/store/apps/details?id=com.nmdp.webView&hl=en#$market://search.

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

- In FY 2019, the donor registry added 1,556,885 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. Of these newly added registrants, 156,836 (10 percent) self-identified as belonging to an underrepresented racial or ethnic population, which is a decrease from the 181,857 self-identifications made in 2018.
- By the end of FY 2019, there were over 22 million volunteer adult bone marrow registrants. More than 3.9 million of the registrants (approximately 18 percent) self-identified as belonging to an underrepresented racial or ethnic population.[15]

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[16] 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 increased by 4 percent in FY 2019 over FY 2018, as shown in Table 4.

[15] Total number of registrants varies 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.
[16] The Stem Cell Therapeutic and Research Act of 2005, 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.

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

| Race/Ethnicity | FY 2018 | FY 2019 | Percent Change |
|---|---|---|---|
| American Indian or Alaska Native | 33 | 12 | -64% |
| Asian | 253 | 270 | 7% |
| Black or African American | 304 | 274 | -10% |
| Multi-racial | 7 | 1 | -86% |
| Native Hawaiian or Other Pacific Islander | 11 | 13 | 18% |
| Unknown+ | 1,402 | 1,382 | -1% |
| White | 4,105 | 4,404 | 7% |
| Hispanic or Latino* | 384 | 434 | 13% |
| **Total** | **6,499** | **6,790** | **4%** |

**Data Source:** NMDP finance department.
#Data in this report may change due to delayed data responses and result in the number of transplants reported to vary from prior year reports.
~ The number of NCBI CBUs declined from FY 2018 to FY 2019 due to the increasing use of alternative therapies. In particular, haploidentical transplants (use of blood stem cells from a donor who is biologically related to the recipient-patient), are on the rise. As the NCBI's diverse inventory of CBUs grows, it will continue to remain key in servicing an increasing number of patients. Underrepresented racial and ethnic populations account for over 63 percent of the CBUs collected. HRSA will continue to monitor and assess trends in cord blood transplantation and will adjust transplant targets accordingly.
+ Unknown reflects those who did not provide race and ethnicity data. This is common when working with international registries where capturing data by race and ethnicity does not occur.
*Hispanic or Latino may be any race.

# 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 information about whether survival rates among the centers in the CWBYCTP network are above expectation, below expectation, or as expected. Transplant centers may also use the report to develop quality improvement initiatives. Results of the 2019 Center-specific Survival Analysis Report were

made available to centers, insurance companies, and patients through the NMDP website[17] in December 2019. Extensive transplant and patient outcome data are available online.[18]

# 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 the CWBYCTP donor registry. CBBs may make donated CBUs available for research if they are not suitable for clinical transplantation. The costs to recruit, collect, test, cryopreserve, and make CBUs available for listing through CWBYCTP varies by CBB.

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 last 5 years of the NCBI Program's appropriations and funding history.

[17] The NMDP website is available at https://bethematch.org/tcdirectory/search/. Please view in a Google Chrome, Safari, Firefox, or Edge browser.
[18] More information about transplant outcomes is available at: https://bloodstemcell.hrsa.gov/data/donation-and-transplantation-statistics.

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

| Fiscal Year* | Appropriation+ | Total Contract Award |
|---|---|---|
| 2015 | $11,266,000 | $10,404,320 |
| 2016 | $11,266,000 | $10,426,197 |
| 2017 | $12,266,000 | $11,329,136 |
| 2018 | $15,266,000 | $14,239,399 |
| 2019 | $16,266,000 | $15,194,125 |
| **Total** | $66,330,000 | $61,593,177 |

**Data Source:** Internal HRSA financial information.
+ Secretary's Transfers and administrative costs account for differences between appropriations and total contract awards.
*This table reflects a 5-year look back at the total appropriations by FY; however, appropriations for the program, currently known as the National Cord Blood Inventory, were first received in FY 2004.

From the FY 2004 inception of the NCBI Program through FY 2019, HRSA awarded 23 NCBI Program contracts to 13 different contractors. Figure 2 identifies organizations holding an NCBI Program contract as of the end of FY 2019. The figure also shows the geographic distribution of NCBI Program contractors. Geographic dispersion not only ensures the 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.

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


**Data Source:** Figure created by HRSA with publicly available information. As of the end of FY 2019, HRSA contracted with 13 CBBs 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, Bloodworks, 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).

# NCBI Program Accomplishments and Statistical Highlights

As of September 30, 2019, funds awarded from FY 2015 through FY 2019 contracted for 31,050 CBUs (see Table 6). Since 2015, approximately 48 percent (2,584) of the 5,394 CBUs (NCBI and non-NCBI) total CBU shipments through the CWBYCTP used CBUs selected from the NCBI CBU inventory (see Table 7).

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 116,000 CBUs for a wide variety of research endeavors.

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. 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 during the past 5 years. CBUs from underrepresented racial and ethnic populations continue to account for over 60 percent of the CBUs contracted through the NCBI Program.

As shown in Table 7, the number of CBUs released for transplant has decreased since FY 2016, primarily due to 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. A secondary factor in the reduction in use of cord blood is the cost related to the price of donor grafts and length of hospitalization after transplant. Despite this recent decrease in CBU usage, patient access to potential life-saving treatments has not decreased. HRSA will continue to monitor and assess trends in cord blood transplantation and share insights in future reports.

During FY 2019, HRSA released a request for proposal for NCBI and awarded five new contracts to the following contractors: Bloodworks, Cleveland Cord Blood Center, LifeSouth Community Blood Centers, Inc., University of Texas MD Anderson Cancer Center, and New York Blood Center, Inc. The amount awarded in FY 2019 was $15,194,125 for 4,585 contract CBUs; 70.7 percent of the CBUs will be from donors from underrepresented racial and ethnic populations.

**Table 6: Contracted National Cord Blood Inventory CBUs by Race/Ethnicity[&~]**

| Fiscal Year[Δ] | Asian | AI/AN[+] (2007-2018) | Black or AA[*] | Hispanic or Latino[^] | Multi-racial (2007-2018) | NH/PI[#] (2007-2018) | White | Multi-race, AI/AN, NH/PI[%] (2019) | Totals |
|---|---|---|---|---|---|---|---|---|---|
| 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 |
| 2019 | 301 | — | 679 | 1,701 | — | — | 1,342 | 562 | 4,585 |
| **Total** | **1,948** | **5** | **5,420** | **8,979** | **4,351** | **12** | **9,773** | **562** | **31,050** |
| **% of Total** | **6.3%** | **0.02%** | **17.5%** | **28.9%** | **14.0%** | **0.04%** | **31.5%** | **1.8%** | **100.0%** |

**Data Source:** Internal HRSA information.

[&] Data in this table reflects a 5-year history, which represents only a subset of the cumulative number of CBUs referenced elsewhere in this report or prior reports.

[~] 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 vary from prior year reports. The next report will include updated information.

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

[+] American Indian or Alaska Native

[*] Black or African American

[^] Hispanic or Latino may be any race.

[#] Native Hawaiian or other Pacific Islander

[%] FY 2019 contracted NCBI CBUs combined three race and ethnicity categories, Multi-racial, AI/AN, and NH/PI, that were separated in previous NCBI-contracted CBUs. In 2019, the total of 4,585 included the 562 for the abovementioned combined race and ethnicity categories. The quantity is smaller because HRSA awarded new contracts in FY 2019. The new contracts resulted in new and higher NCBI CBU costs per unit.

**Table 7: CBUs Released for Transplantation for 2015-2019^**

| Fiscal Year | NCBI-funded CBU Shipments+ | Total CBU Shipments* |
|---|---|---|
| 2015 | 609 | 1,393 |
| 2016 | 529 | 1,154 |
| 2017 | 494 | 1,050 |
| 2018 | 493 | 949 |
| 2019 | 459 | 848 |
| **Total** | **2,584** | **5,394** |

**Data Source:** NMDP – Finance.
^ Data in this table reflects a 5-year history, which represents only a subset of the cumulative number of CBUs referenced elsewhere in this report or prior reports.
+ Data in this report may change due to delayed data responses. The next report will include updated information.
* Includes NCBI and non-NCBI CBUs.

# Highlights from FY 2019 Demonstration Projects

In FY 2016, the Office of the Assistant Secretary for Health with the RAND Corporation conducted a study on the financial sustainability of the public cord blood banking industry.[19] In direct response to recommendations made from that study, the CWBYCTP asked the cord blood community, via the NMDP's Cord Blood Advisory Group, to identify priority areas for potential future demonstration projects or special studies to increase cord blood utilization. The Cord Blood Advisory Group includes representatives from CBBs and laboratories, transplant physicians, researchers, and others from across the public cord blood community. Below are the outcomes of the projects.

## Explore CBU Selection Options for Transplant Centers with Difficult Donor Searches

HRSA designed this ongoing demonstration project to provide transplant centers with cord blood selection information and advice from physicians experienced in cord blood selection and transplantation. This project assists with minimizing delays in time to transplant and identifying individuals with difficult searches (i.e., those unlikely to have a fully matched adult donor). Through the demonstration project, interested transplant centers with limited experience in selecting suitably matched CBUs receive search strategy assistance if they do not have a fully matched adult donor option and cannot identify a suitably matched CBU.

During FY 2019, four transplant centers received cord blood consultations on behalf of four patients. The search strategy assistance demonstration program resulted in two of the four

[19] A summary of these findings is in the FY 2018 CWBYCTP Report to Congress available at https://bloodstemcell.hrsa.gov/sites/default/files/bloodstemcell/about/legislation/fiscal-year-2018-annual-progress-report.pdf.

patients, whose donor searches doctors initially viewed as futile, receiving cord blood transplants.

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

In FY 2019, the CWBYCTP provided support to NCBI contractors with existing agreements at Grady Hospital (Atlanta, Georgia) and the Memorial Hermann Hospital System (Houston, Texas) to support CBBs in collecting 560 CBUs from underrepresented racial and ethnic populations. This support included funding to hire and train the necessary staff that enhance collections, such as cord blood bank liaisons, cord blood collectors, quality assurance specialists, data entry specialists, and logistics coordinators.

## Identified Demonstration Projects for FY 2020

In FY 2020, HRSA will continue to consider the recommendations from the aforementioned RAND study to identify priority areas for potential future demonstration projects or special studies. For example, HRSA will continue to support 2 previously funded demonstration projects with the aim of (1) exploring CBU selection options for transplant centers with difficult donor searches and (2) supporting CBBs with existing collection efforts at 3 birthing centers with high birth rates among underrepresented racial and ethnic populations to rapidly add 200 CBUs.

In addition, HRSA will broaden and support clinical expertise in cord blood transplantation through the development and adoption of a Cord Blood Transplant Standard of Care Package – step-by-step guidelines for selection, patient and product preparation, infusion, graft versus host disease and infectious disease prophylaxis, and follow-up care. HRSA will also provide funding to tissue type more CBUs at a higher resolution level with the aim of making the matching of the CBUs to the patient more precise, thus assisting transplant centers' more rapidly evaluate and select a CBU.

Another project is to draft contingency planning guidelines to mitigate the loss of CBUs in the event of financial instability. HRSA intends these guidelines to provide CBBs with items to consider should they experience a disruption in business operations.

HRSA will provide any major findings resulting from these special projects in a future report to Congress.

# Stem Cell Therapeutic and Research Reauthorization Act Report to Congress

The Stem Cell Therapeutic and Research Reauthorization Act of 2015 (P.L. 114-104, section 2(c)) called for an additional report that required that the Secretary of HHS, working with the National Institutes of Health, Food and Drug Administration (FDA), and others, provide the United States Congress with a report regarding the appropriateness of the inclusion in the CWBYCTP of adult stem cells and birthing tissues as new types of therapies for patients. HHS appropriately submitted the mandated report, satisfying the requirement.

The CWBYCTP currently facilitates access to the following potentially life-saving therapies that the medical community generally accepts as standard of care: stem cells derived from blood, bone marrow, or cord blood used for hematologic or immunologic reconstitution. These uses of stem cells have been incorporated into professional guidelines for the management of many different diseases, and there are several cord blood products approved[20] by the FDA for use in hematologic and immunologic reconstitution.

Stem cells derived from allogeneic (cells from one individual to another) and autologous (cells from one's own body) bone marrow and peripheral blood, cord blood, as well as stem cells derived from adipose tissue and other sources, such as birthing tissues, are being investigated for the treatment of a wide variety of different diseases and conditions ranging from arthritis to neurologic disorders to cardiac diseases. In contrast to clinical evidence supporting the use of stem cells derived from bone marrow, peripheral blood, or cord blood for hematologic and immunologic reconstitution, clinical trials have yet to demonstrate through sufficient evidence the safety and efficacy of adult stem cells or any stem cells derived from birthing tissues for the treatment of any non-hematologic or non-immunologic conditions.

At this time, adult stem cells and birthing tissues, as discussed above, are not FDA approved products for any use and are considered investigational for those intended uses. Therefore, the report does not support the inclusion of such investigational uses into the CWBYCTP at this time but rather encourages reevaluation as the science advances.

Based on the considerations discussed above, and the evolution of the field of stem cell-based therapies, HHS made the following recommendations regarding the proposed criteria for inclusion of new cellular therapies in the CWBYCTP:

1. The CWBYCTP should include only those adult stem cell and birthing tissue products, including those with new uses outside of hematologic or immunologic reconstitution, that:
    a. are utilized as treatments for serious or life-threatening conditions,

[20] FDA approval to market a biological product is granted by issuance of a biologics license under section 351 of the Public Health Service Act, 42 USC 262. For the purposes of this report, the terms "approval" and "licensure" are used interchangeably with respect to biological products.

   b. require donor matching if appropriate, and
   c. have been demonstrated to be safe and effective, as evidenced by FDA approval, or if FDA approval is not required, through adoption as a standard of care.
2. Based on the criteria above, HHS does not recommend the inclusion of stem cells derived from blood, bone marrow, or cord blood for uses other than hematologic or immunologic reconstitution, and does not recommend stem cells from birthing tissues or other tissues for any use at this time.
3. As the science advances and development of new classes of cell-based products meet regulatory approval standards for safety and efficacy, it may be appropriate to include such products in the CWBYCTP. Therefore, reevaluation by HRSA, the National Institutes of Health, and FDA (in conjunction with appropriate expert consultation) of the status of adult stem cells and birthing tissues for potential inclusion in the CWBYCTP is recommended on a periodic basis (every 2 to 3 years or as needed), with issuance of a report on the outcomes of these evaluations when relevant.

# 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 HHS 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 held a virtual meeting on September 10, 2019. The Advisory Council received information on the Report to Congress on Appropriateness of Blood Stem Cells and Birthing Tissue for the Potential Inclusion in the C.W. Bill Young Cell Transplantation Program,[21] which HRSA submitted in September 2019. The ACBSCT also received several presentations on current efforts to expand utilization of cord blood and discussed the impact of FDA screening guidance on CBBs. The ACBSCT approved one recommendation related to this last topic, which reads:

> *"Based on current data and reduction in observed and confirmed Zika infection, ACBSCT recommends that HHS review the current FDA Recommendations for Reducing the Risk of Zika Virus Transmission by Blood and Blood Components as the guidance relates to utilization of cord blood."*

The ACBSCT has made 32 recommendations[22] to the Secretary of HHS. 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

[21] The report is available at https://bloodstemcell.hrsa.gov/about/legislation/reports-congress.
[22] The ACBSCT recommendations are available at http://bloodcell.transplant.hrsa.gov/about/advisory_council/recommendations/index.html.

roster, agendas for upcoming meetings, and meeting summaries are available on the CWBYCTP website.[23]

# 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 13 years, the CWBYCTP listed over 101,120 CBUs on the donor registry. With over 22 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.

[23] Information is available at http://bloodcell.transplant.hrsa.gov.