



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

REPORT TO CONGRESS

**Fiscal Year 2020 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) 2020 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, 2019, through September 30, 2020.

The report provides background information about each program, describes their structure and operation, and provides statistical information on the number of bone marrow donor registrants and collected cord blood units (CBUs), along with other data. Unless otherwise noted, the information presented is from FY 2020. This is an update to the FY 2019 report, which included information through September 30, 2019.

The purpose of CWBYCTP is to increase the number of bone marrow and cord blood transplants for recipients matched to biologically unrelated donors. Every year, approximately 18,000 patients are diagnosed with life-threatening blood cancers or other diseases for which a blood stem cell transplant may be their best or only hope for a cure. Often, the ideal donor is a suitably matched family member, but only 30 percent of people have a fully matched relative. 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. Both the CWBYCTP and the NCBI have enabled thousands of transplant candidates, who lack suitably matched relatives, to explore viable options and identify matched unrelated blood stem sources (e.g., bone marrow, cord blood, etc.).

In its mission to increase access to HSCT transplants, CWBYCTP also addresses health inequities for both underserved populations and individuals from underrepresented racial and ethnic populations (e.g., American Indian or Alaska Native, Asian, Black or African American, Multi-racial, Native Hawaiian or Other Pacific Islander, Hispanic or Latino). One way the CWBYCTP accomplishes this is by providing oversight of contracts to ensure adequate resources are leveraged for patient advocacy support, public and professional education, data collection, donor recruitment, and expansion of the size and diversity of the donor registry. This ensures 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.

By the end of FY 2020, there were over 23 million volunteer adult bone marrow registrants listed through the CWBYCTP. Of those registrants, there were 3.9 million (17 percent), self-identified as belonging to an underrepresented racial or ethnic population. Because patients from underrepresented populations find suitably matched unrelated donors at a rate lower than that of White, non-Hispanic patients (ranging from 23 percent to 57 percent versus 77 percent, respectively), the program continues to seek to grow the registry, particularly registrants from underrepresented populations.

The NCBI contracts with cord blood banks to purchase 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 CBUs, which increases access to transplantation. The NCBI continues to grow and diversify with over 104,500 NCBI CBUs available on the donor registry through CWBYCTP. More than 60 percent of the total NCBI CBUs are from underrepresented racial and ethnic populations.

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. The ACBSCT held virtual meetings on April 27, 2020, and September 25, 2020, and made one recommendation encouraging HRSA's continued support of the collection of NCBI CBUs and optimizing the utilization of cord blood. During the meetings, the ACBSCT received presentations and updates on the impact of the novel coronavirus disease 2019.



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Acronym List

ACBSCT	Advisory Council on Blood Stem Cell Transplantation
ASPR	Office of the Assistant Secretary for Preparedness and Response
ASTCT	American Society for Transplantation and Cellular Therapy
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
COVID-19	Coronavirus Disease 2019
CWBYCTP	C.W. Bill Young Cell Transplantation Program
FY	Fiscal Year
HHS	Department of Health and Human Services
HLA	Human Leukocyte Antigen
HRSA	Health Resources and Services Administration
HSCT	Hematopoietic Stem Cell Transplants
MCW	Medical College of Wisconsin
MDS	Myelodysplastic Syndrome
NCBI	National Cord Blood Inventory
NMDP	National Marrow Donor Program
OMB	Office of Management and Budget
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

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 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. It plays a vital role in addressing health inequities by expanding access to hematopoietic stem cell transplants (HSCT) to those from underrepresented racial and ethnic populations (American Indian or Alaska Native, Asian, Black or African American, Multi-racial, Native Hawaiian or Other Pacific Islander, Hispanic or Latino). 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 opportunity 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 HSCT. CWBYCTP also 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.

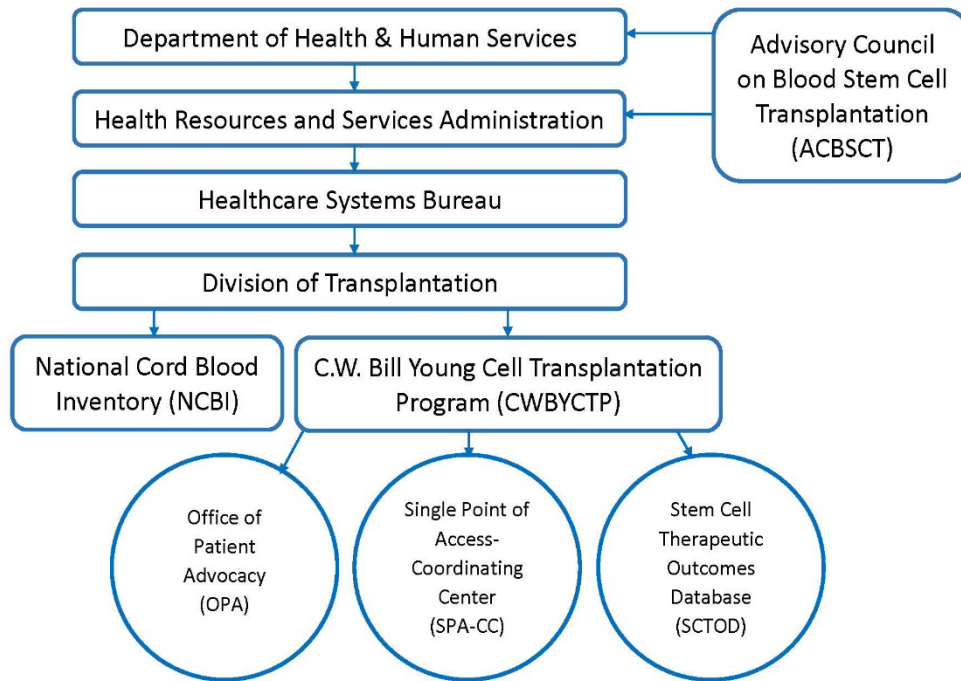
III. CWBYCTP Overview

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 contractual functions (Bone Marrow Coordinating Center (BMCC), Cord Blood Coordinating Center (CBCC), Single Point of Access (SPA), Office of Patient Advocacy (OPA), and Stem Cell Therapeutic Outcomes Database (SCTOD)).¹ 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 in November 2016, HRSA determined that restructuring the contracts from four contracts to three contracts by combining some of the functions would be a more streamlined and beneficial approach for the government, and awarded the three major contracts in September 2017. The following is a description of the three current major contracts:

- The Single Point of Access-Coordinating Center (SPA-CC) contract, which is a combination of the single point of access and bone marrow and cord blood coordinating 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 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 electronic access.
- The 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 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 recipient samples for research and the collection and analysis of data on clinical outcomes of HSCT recipients and blood stem cell products.

¹ In fiscal year (FY) 2020, the Medical College of Wisconsin (MCW), the parent organization of the Center for International Blood and Marrow Transplant Research (CIBMTR), was the contractor for the SCTOD contract, and the National Marrow Donor Program (NMDP) was the contractor for the OPA and SPA-CC contracts.

Figure 1: CWBYCTP and NCBI Program



Data Source: Internal HRSA information.

Table 1: Funding for the CWBYCTP Contracts for 2016 to 2020⁺*

FY	Appropriations	Single Point of Access-Coordinating Center[^]	Office of Patient Advocacy[^]	Stem Cell Therapeutic Outcomes Database[^]	Total Program Contracts[^]
2016	\$22,109,000	\$14,352,558	\$781,302	\$4,156,111	\$19,289,971
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
2020	\$30,009,000	\$21,804,584	\$877,293	\$4,601,550	\$27,283,427
Total	\$122,945,000	\$84,718,960	\$4,140,119	\$21,951,724	\$110,810,803

Data Source: Internal HRSA financial information.

+Secretary’s Transfers and administrative costs account for differences between appropriations and total program contracts awarded.

*Appropriations for FY 2016 and prior implemented a contracting structure that included the following four contracts: BMCC, CBCC, SPA/OPA, and SCTOD. FY 2016 appropriations for SPA-CC included \$12,415,360 for the BMCC function and \$1,937,198 for CBCC function.

[^]Appropriations for FY 2017 and after, implemented a more streamlined contracting structure that includes three contracts instead of four. The BMCC, CBCC, and SPA functions combined to form the SPA-CC contract.

CWBYCTP Statistical Updates

Every year, approximately 18,000 patients are diagnosed with life-threatening blood cancers or other diseases for which a blood stem cell transplant may be their best or only hope for a cure. Often, the ideal donor is a suitably matched family member, but only 30 percent of people have a fully matched relative. The other 70 percent, or approximately 12,600 people, often search for a matched unrelated adult donor or umbilical cord blood unit. Adding volunteer adult bone marrow registrants and high-quality, diverse CBUs to the CWBYCTP helps individuals without a matched family member identify other potential matched blood stem cell sources. CWBYCTP serves individuals in need of unrelated blood stem cell transplantation and works to ensure 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 chance of finding a suitably matched unrelated donor through the CWBYCTP varies by race and ethnicity. For example, the chance of finding a suitably matched unrelated donor varies from 23 percent for Black/African American; 41 percent for Asian/Pacific Islander; 46 percent for Hispanic/Latino; 57 percent for American Indian or Alaska Native; to 77 percent for White, non-Hispanic.

The total number of volunteer adult bone marrow registrants is currently over 23 million, with more than 3.9 million (17 percent) self-identified as belonging to an underrepresented racial or ethnic population. A total of 265,862 new registrants were added to the CWBYCTP in FY 2020 (see Table 2) and 42 percent of those self-identified as belonging to an underrepresented racial or ethnic population, an increase over the 37 percent in FY 2019. Although the overall number of donor registrations decreased in FY 2020, the percentage of registrants self-identifying within each underrepresented racial or ethnic population remained relatively flat with no statistically significant changes. Table 2 data reflects that the Hispanic or Latino group with a 4.33 percentage point increase and the White group with a 5.23 percentage point decrease were the only two categories showing significant changes in donor composition. It is widely believed within the transplant community that the unanticipated public health emergency resulted in a decrease in the overall number of volunteer adult donors added. Details regarding those challenges are outlined under the “COVID-19 Impact on CWBYCTP” section.

Table 2: Number of Registrants Added to the CWBYCTP by Race/Ethnicity⁺

Race/Ethnicity	FY 2019	Percentage of Total Number of Registrants Added	FY 2020	Percentage of Total Number of Registrants Added
American Indian or Alaska Native	1,031	0.30%	693	0.26%
Asian	12,913	3.82%	12,242	4.60%
Black or African American	9,325	2.76%	10,927	4.11%
Multi-racial	42,297	12.51%	30,002	11.28%
Native Hawaiian or Other Pacific Islander	324	0.10%	215	0.08%
Unknown	806	0.24%	729	0.27%
White	212,708	62.91%	153,359	57.68%
Sub-Total	279,404		208,167	
Hispanic or Latino (subset of total above)	58,723	17.37%	57,695	21.70%
Total (race plus ethnicity)	338,127		265,862	

Data Source: NMDP data.

⁺Unknown reflects people 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.

[^]Individuals cannot choose more than one racial/ethnic category in the data used as the source for this table. As such, someone who is Black or African American and Latino have the option of choosing either “Black or African American” or “Latino” as a racial category, but not both. In short, only one category can be selected, which prevents double counting.

Through the CWBYCTP, physicians working on behalf of patients can search for volunteer adult donors or CBUs as potential matches for a transplant. The number of unrelated blood stem cell transplants facilitated by the CWBYCTP decreased by 2 percentage points from FY 2019 to FY 2020 as shown in Table 3. The percentage of transplants facilitated across racial categories remained relatively flat with a nominal percentage rate change of less than 2 percent across all tracked categories. It is widely believed in the transplant community that the unanticipated public health emergency resulted in a decrease in the number of blood stem cell transplants performed in FY 2020. Details regarding challenges encountered are outlined in the “COVID-19 Impact on CWBYCTP” section.

Table 3: Number of Transplants Facilitated by the CWBYCTP by Race/Ethnicity^{†*^}

Race/Ethnicity	FY 2019	Percent of Total Number of Transplants Facilitated	FY 2020	Percent of Total Number of Transplants Facilitated
American Indian or Alaska Native	12	0.19%	35	0.57%
Asian	265	4.19%	218	3.53%
Black or African American	266	4.20%	266	4.30%
Multi-racial	1	0.02%	9	0.15%
Native Hawaiian or Other Pacific Islander	12	0.19%	9	0.15%
Unknown	1,425	22.53%	1,291	20.88%
White	4,345	68.68%	4,356	70.44%
Sub-Total	6,326		6,184	
Hispanic or Latino (subset of total above)	3	0.05%	9	0.15%
Total (race plus ethnicity)	6,329		6,193	

Data Source: NMDP data.

+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.

*+Unknown reflects people 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.

^ Individuals cannot choose more than one racial/ethnic category in the data used as the source for this table. As such, someone who is Black or African American and Latino have the option of choosing either “Black or African American” or “Latino” as a racial category but not both. In short, only one category can be selected, which prevents double counting.

Transplant Survival Rates

Because CWBYCTP supports individuals from diagnosis to post-transplant, the program establishes goals not only for the number of transplants facilitated but also for the outcomes of these transplants. The FY 2020 target is a rate of 69 percent patient survival at 1 year, post-transplant for matched unrelated blood stem cell transplants. The 1-year survival of first allogeneic HSCT is an outcome generally accepted as a measure of performance by the HSCT centers, which can be obtained via the reporting tool for the Center-Specific Survival Analysis²

² Information about the Center-Specific Survival Analysis is available at <https://bethematch.org/tcdirectory/search/> (not supported in Internet Explorer).

produced on behalf of the CWBYCTP. Due to the nature of the data, there are inherent delays in collecting, reporting, and analyzing data from transplant centers on survival outcomes. Additionally, to accurately report the probability of survival at 1 year following HSCT, all patients must be followed for at least a year after receiving a transplant. Allowing for adequate follow-up and reporting by transplant centers, along with compilation and analysis of the data, creates a lag in reporting of survival outcomes; therefore, FY 2020 transplant outcomes data, including the probability of 1-year survival, will be reported in the FY 2022 report.

For this FY 2020 report, transplant outcomes for FY 2018 were the latest full dataset available and are reported and compared to FY 2017 data (see Table 4). The 69 percent goal was surpassed in FY 2017 and FY 2018. Improvements in donor-recipient matching over time and improvements in supportive care have contributed to continued improvements in survival for patients transplanted in FY 2018, compared to FY 2017, across the broad indications for HSCT.

Table 4: 1-year Post-transplant Survival Rate (U.S. Transplant Centers) for 2017 and 2018

Donor Type	FY 2017	FY 2018
Unrelated	71%	74%
Matched Related	78%	80%
Mismatched Related	72%	75%

Data Source: CIBMTR data.

Centers for Medicare & Medicaid Services Coverage with Evidence Studies

To address concerns regarding lack of access to HSCT for some individuals diagnosed with Myelodysplastic Syndrome (MDS), the American Society for Transplantation and Cellular Therapy (ASTCT - formerly known as the American Society of Blood and Marrow Transplantation), 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 December 2010 to provide Coverage with Evidence Development (CED) for MDS. CMS’s decision led to the completion of a study that leveraged the reporting requirements of the SCTOD and met the criteria for the CED mechanism. Ensuring coverage under the CED mechanism increased the number of patients with MDS receiving transplants in the United States.

The above-mentioned partners, supported by recommendations from the ACBSCT, advocated for additional coverage decisions that led to opportunities for three additional CED studies, resulting in a total of four active studies conducted under CMS’ CED mechanism. Information about the disease indications, when the studies were open to patient enrollment, and the number of patients enrolled through the end of FY 2020 are provided (see Table 5).

These studies, which are approved under CMS’s CED paradigm on the condition that they are furnished in the context of approved clinical studies or with the collection of additional clinical data are leveraged to improve access to HSCT for older Americans with appropriate indications for transplantation.

Table 5: Number of Patients 65 and Older Enrolled in CMS CED Studies through September 30, 2020

Disease Indication	Study Opened	Unrelated Donor	Related Donor	Total
Myelodysplastic Syndrome	December 2010	3,243	1,714	4,957
Myelofibrosis	December 2016	198	83	281
Multiple myeloma	July 2017	9	11	20
Sickle cell disease	November 2017	2	5	7
Total		3,452	1,813	5,265

Data Source: CIBMTR data.

CWBYCTP Professional Education and Outreach Highlights

The novel coronavirus disease 2019 (COVID-19) pandemic impacted the delivery of education programs to medical professionals. Prior to COVID-19, many educational programs were delivered in person and recorded for online access. Due to the pandemic, beginning in March 2020, all national and regional conferences were conducted virtually, if at all. CWBYCTP provided professional education and outreach virtually through online meetings and pre-recorded sessions, described below.

Transplant Referral Timing Guidelines and Resources

A timely referral is critical to patients receiving an HSCT. The CWBYCTP educates those within the blood stem cell community on the importance of both transplant referral timing and early human leukocyte antigen (HLA) typing for patients. These meetings included presenting peer-reviewed research “Rapid Donor Identification Improves Survival in High-Risk First-Remission Patients With Acute Myeloid Leukemia.”³ Furthermore, Transplant Referral Timing Guidelines were developed in 2019 through a comprehensive review and research process in collaboration with ASTCT. The guidelines are available in print, online, and mobile app versions.⁴

³ Pagel JM, Othus M, Garcia-Manero G, et al. “Rapid Donor Identification Improves Survival in High-Risk First-Remission Patients With Acute Myeloid Leukemia,” *JCO Oncology Practice*, 2020; 16:6, e464-e475.

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

Education Program for Health Professionals

CWBYCTP offers 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 transplantation. For example, CWBYCTP developed healthcare professional education programs focusing on the use of HSCT in treating sickle cell disease. The “Advances in Bone and Marrow Transplantation for Sickle Cell Disease” program was provided through a webinar and also offered online for continuing education credits. To reach hematologists and oncologists nationally, the content was provided for inclusion in the American Society of Hematology’s Sickle Cell Disease Coalition newsletter. The content was published in the March, April, and May 2020 issues to educate subscribers about sickle cell disease resources available to patients and caregivers. A “Sickle Cell Disease: Exploring Treatment Options and Psychosocial Care” webinar was co-hosted with the Sickle Cell Transplant Advocacy & Research Alliance through a webinar in March 2020.

CWBYCTP Public Educational Resources and Services

Navigation Services and Education Resources

CWBYCTP provides patient navigation services and develops resources to guide patients through all phases of transplantation. Beginning in October 2019, the SPA-CC contractor implemented a proactive outreach program, reaching out to patients when a preliminary search of the registry was initiated. This new program includes calls to identify patient concerns and to help remove barriers to transplant through referral to the SPA-CC contractor. Also, an information packet was mailed to all potential patients to inform them of the services and resources available to them.

To address patient concerns during the COVID-19 pandemic, CWBYCTP developed resources to support patients, including two pre-HSCT and post-HSCT COVID-19 fact sheets specifically designed for patients. Both fact sheets are available in English and Spanish. CWBYCTP proactively delivered COVID-19 fact sheets to patients with a planned NMDP-facilitated transplant during the previous 12 months. In addition, a webinar titled “Coping and Coronavirus” was held to provide information and answer patient questions about COVID-19.

Summary of Published and Unpublished Studies for SCTOD

The SCTOD contractor has developed, conducted, and published research studies in the following relevant areas:

- Studies evaluating the optimal selection, harvesting, and processing of an unrelated donor graft,
- Studies evaluating the optimal choice and handling of a cord blood unit for transplantation,

- Studies comparing alternative donor and graft sources for patients without HLA identical sibling donor(s),
- Studies evaluating optimal patient selection and treatment strategies for unrelated donor HSCT,
- Studies evaluating optimal patient selection and treatment strategies for cord blood HSCT,
- Studies evaluating access to care, and
- Studies evaluating quality of life and late effects of allogeneic HSCT.

The SCTOD contractor's portfolio includes more than 165 unpublished studies in progress. The completed studies resulted in 74 peer-reviewed publications⁵ involving blood stem cell transplantation. The following are several of the journals that published articles based on completed studies:

1. *JAMA Oncology*,
2. *Lancet Haematology*,
3. *Journal of Clinical Oncology*,
4. *Leukemia*,
5. *Blood*,
6. *British Journal of Haematology*,
7. *Cancer*,
8. *Biology of Blood Marrow Transplantation*,
9. *Blood Advances*, and
10. *Bone Marrow Transplantation*.

Eighteen plain language or non-scientific summaries were published,⁶ surpassing the SCTOD contractor's annual goal of eight summaries designed specifically for patient use. This helps fulfill the legislative requirement to make relevant scientific information available to the public.

SPA-CC Support for Cord Blood Expansion

Highlights from FY 2020 Demonstration Projects

The Stem Cell Therapeutic and Research Act of 2005, P.L. 109-129, as amended by P.L. 111-264 (section 379(d)(2)(A)(iv) of the Public Health Service Act) and P.L. 114-104, includes a requirement in section 2 which states, in part:

⁵ The CIBMTR Publication List is available at <https://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx>.

⁶ The CIBMTR Study Summaries for Patients is available at <https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/pages/index.aspx>.

“...support and expand new and existing studies and demonstration and outreach projects for the purpose of increasing cord blood unit donation and collection from a genetically diverse population and expanding the number of cord blood unit collection sites partnering with cord blood banks receiving a contract under the National Cord Blood Inventory program...”

Here are summaries of ongoing projects:

Explore CBU Selection Options for Transplant Centers with Difficult Donor Searches

This ongoing demonstration project provides 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 can receive search strategy assistance if they do not have a fully matched adult donor option and cannot identify a suitably matched CBU.

On behalf of nine patients, seven transplant centers received cord blood consultations beyond the assistance usually provided by NMDP’s Immunogenetic Specialists. This program resulted in four of the nine patients, whose donor searches doctors initially viewed as futile, receiving cord blood transplants.

Create a Cord Blood Transplant Standard of Care Package to Support Hematopoietic Transplant using Cord Blood at Two Key Domestic Transplant Centers

HRSA supported the development of a plan to provide support to physicians who do not regularly utilize cord blood as a graft source when performing hematopoietic transplants. From this project, clinicians with extensive cord blood transplant experience created “The Cord Blood Transplant Guidelines,” and it will be used as the fundamental component of the Standard of Care Package.

These guidelines are structured in a question and answer format and contain eight chapters, each of which will undergo an approval process by the ASTCT guidelines committee and executives, then will be uploaded to the ASTCT Cord Blood Special Interest Group webpage and ultimately to an educational app. Once all the chapters are published, permission may be requested from ASTCT and the publishers of the *Biology of Blood and Marrow Transplantation* journal to create a compendium for the Standard of Care Package. By the end of FY 2020, two chapters were published, and two others were either in press or in review. The distribution of these chapters and overall guidelines are under discussion and decisions had not been finalized by the closing date of this report.

Cord Blood Contingency Considerations and Templates to Address Transferring a Public Cord Blood Inventory

A 2017 RAND report⁷ noted the potential risks of consolidation within the cord blood banking community and recommended that contingencies for protecting CBUs in case of adverse events, such as bankruptcy, be developed to ensure those CBUs do not become inaccessible. As such, conditions for NCBI-contracted banks were included in a recent HRSA solicitation to ensure the protection of the CBUs funded under the NCBI Program. The solicitation requires that contractors maintain a contingency plan for transferring their cord blood inventory in the event that the contractor can no longer meet contract requirements and for receiving inventory in the event that the contractor receives CBUs from another CBB.

In addition, the SPA-CC collaborated with the cord blood community, via a task force, to create contingency planning guidelines for CBBs to mitigate the loss of CBUs and preserve the national (domestic) inventory in the event of business disruptions. This list of guidelines are ones that CBBs might consider in the event that their financial sustainability is threatened or compromised; to safeguard against destruction, damage, or loss; ensure the existence and availability of CBUs, and protect patient access to donor grafts. The guidelines were finalized in August 2020 and are being reviewed by the cord blood community for adoption. The document was posted and made available through the SPA-CC to NMDP member CBBs through the network website as a resource to assist CBBs in the development of their internal contingency plans.

Provide Support to Three NCBI CBBs in Collecting CBUs from Underrepresented Racial and Ethnic Populations

The CWBYCTP provided support to NCBI contractors Duke University, MD Anderson, and Bloodworks, with existing agreements at Grady Hospital in Atlanta, the Memorial Hermann Hospital System in Houston, and various sites in Seattle, respectively, to support CBBs in collecting 380 CBUs from underrepresented racial and ethnic populations. This support included funding to expand collection site hours and hire and train necessary staff to enhance collections, including CBB liaisons, cord blood collectors, quality assurance specialists, data entry specialists, and logistics coordinators.

Support to CBBs to Perform High-Resolution Tissue Typing of 1,376 Genetically Diverse CBUs

HRSA supported the coordination of high-resolution typing on segments from 1,376 CBUs at 10 CBBs (both NCBI and non-NCBI funded) to improve search quality, enhance matching, and expedite deliverability. This activity aimed to simplify search navigation and prioritize the appearance of CBUs with more complete typing on search reports, which may increase the probability of selection of cord blood as a graft source and mobilize the use of the inventory.

⁷ *Challenges to the Sustainability of the U.S. Public Cord Blood System* is available at https://www.rand.org/pubs/research_reports/RR1898.html

Add an Additional 4,025 Volunteer Adult Donors and Conduct an Additional 4,025 HLA Typing Tests from Underrepresented Racial and Ethnic Populations

HRSA supported efforts to add over 4,000 volunteer adult donors from underrepresented populations. These efforts were implemented through (1) maintaining a two-step registration process, followed by mailing kits to the registrant to increase donor availability, commitment, and follow-through at donation; (2) deploying on 270 college campuses using a newly created account management college strategy, which included leveraging key administrative campus leader contacts and student groups; (3) focusing efforts on patient and donor stories reflecting African American, Asian, and Hispanic individuals on social media with a targeted digital campaign; and (4) creating new strategic partnerships with Black/African-American groups. The HLA typing was supported through contracting with accredited HLA laboratories and monitoring the labs' performance.

Identify and Submit Reports on Blood Stem Cell-Related Donor Observances Observed by the Contractor

The CWBYCTP identified 28 blood stem cell-related observances. Those observances were amplified through various platforms, including media outreach, social media awareness, engagement activities, and physician awareness. Observances included National Blood Donor Month (January), World Cancer Day (February), National Cancer Survivors Day (June), and Sickle Cell Awareness Month (September).

Identified Demonstration Projects for FY 2021

HRSA will continue to consider the recommendations from the aforementioned 2017 RAND study to identify priority areas for potential future demonstration projects or special studies. In FY 2021, HRSA will enhance the professional development of the workforce in the area of cord blood; continue efforts to support CBBs with expanding CBU collection at birthing centers; and support difficult searches as outlined on page 16 of this report, under the “SPA-CC Support for Cord Blood Expansion” section.

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

COVID-19 Impact on CWBYCTP

In March 2020, NMDP (the contractor for the SPA-CC and OPA contracts) notified HRSA of a projected increase in unplanned expenses that were also anticipated to persist throughout the remainder of FY 2020 because of the COVID-19 pandemic. The areas that primarily caused the unanticipated increase in expenses, included donor recruitment and retention, cryopreservation, and donor and courier travel.

As such, NMDP requested additional funding of over 5 million dollars, which had already been appropriated for within the CWBYCTP for FY 2020, to use for the SPA-CC contract March 1, 2020, through September 29, 2020. HRSA's collaborative efforts and financial support provided the following:

Support for Network Partners

HRSA provided support to recruitment centers impacted due to closures and canceled recruitment drive events beginning in March 2020. This funding ensured recruitment centers remained staffed during this interim period thus allowing recruiters to resume recruitment activities as soon as reasonably practicable.

International Air Support for Transplantation

Due to COVID-19 and its impact on international air transportation, in April 2020 HRSA began working with the Office of the Assistant Secretary for Preparedness and Response (ASPR) to address NMDP's growing concerns over its continuing ability to transport lifesaving blood stem cell products internationally. Products from foreign donors play a key role in meeting the needs of patients in the United States, therefore, devising a solution was imperative. HRSA and ASPR's collaborative efforts led to facilitating a partnership with NMDP and Lockheed Martin, which offered in-kind air support to HHS. During periods of limited international air access, Lockheed Martin was able to successfully fly NMDP couriers with life-saving products from Germany and Canada saving multiple lives. For ease of coordination, Germany was used as the hub for products collected throughout Europe thus limiting further logistical challenges.

Data Collection to Support Future Analyses of COVID-19 Impact on HSCT

The Medical College of Wisconsin (MCW) realized when the COVID-19 pandemic started in March that it would be essential to collect information from HSCT centers to properly evaluate the impact of the pandemic on HSCT methods, practices, and outcomes in the United States and on the CWBYCTP. The contractor and HRSA worked collaboratively to revise the data collection forms used to support the CWBYCTP to include COVID-19 related data fields. HRSA received approval from the Office of Management and Budget (OMB) by the first week of April and data collection began immediately thereafter. Subsequently, MCW and HRSA collaborated to further revise data collection forms with additional COVID-19 related data fields and received rapid approval from OMB in July 2020. These additional data points began to be collected within a few weeks. Collectively, these adaptive changes to the data collection instruments will allow the CWBYCTP to better evaluate changes in HSCT practices in response to the pandemic (including the impact of processing and cryopreservation mentioned above), potential impacts on access to HSCT, and resulting outcomes at U.S. HSCT centers.

IV. NCBI 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 CBUs to be reimbursed per year, and the agreed-upon racial/ethnic mix of donors (see more details in Table 7). Setting racial/ethnic collection goals helps to ensure that collected CBUs emanate 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 6 shows the previous 5 years of the NCBI Program's appropriations and funding history.

Table 6: Appropriations and Contract Funding History for the NCBI Program for 2016 to 2020+*

Fiscal Year	Appropriation	Total Contract Award
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
2020	\$17,266,000	\$16,221,529
Total	\$72,330,000	\$67,410,386

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 review of the total appropriations by fiscal year; however, appropriations for the program, currently known as the National Cord Blood Inventory, were first received in fiscal year 2004.

From the FY 2004 inception of the NCBI Program through FY 2020, HRSA awarded 28 NCBI Program contracts to 13 different contractors. Figure 2 identifies organizations holding an NCBI Program contract as of the end of FY 2020 and 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 collected and available to help more individuals in need.

Figure 2: NCBI Banks



Data Source: Figure created by HRSA with publicly available information. As of the end of FY 2020, 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 the University of Texas MD Anderson Cancer Center (MD Anderson).

NCBI Program Accomplishments and Statistical Highlights

As of September 30, 2020, funds awarded from FY 2016 through FY 2020 contracted for 29,148 CBUs (see Table 7). Since 2016, approximately 53 percent (2,319) of the 4,339 CBUs (NCBI and non-NCBI) total CBU shipments 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 nearly 50,000 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 and enhanced health equity. 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 7 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 8, 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 the use of cord blood is the cost related to the price of donor grafts and length of hospitalization after transplant. Despite this recent overall decrease in cord blood transplants, patient access to potentially life-saving blood stem cell treatments across racial and ethnic categories remained relatively constant, as outlined in Table 3. HRSA will continue to monitor and assess trends in cord blood transplantation and share insights in future reports.

HRSA provided financial support to the following five NCBI contractors: Bloodworks, Cleveland Cord Blood Center, Duke University, LifeSouth Community Blood Centers, Inc., and University of Texas MD Anderson Cancer Center. In FY 2020, the amount awarded was \$16,221,529 for 4,567 CBUs, and 71 percent of these CBUs were from donors from underrepresented racial and ethnic populations.

Table 7: Contracted NCBI CBUs by Race/Ethnicity for 2016 to 2020^{&~}

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
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
2020	256	—	780	1,330	—	—	1,335	866	4,567
Total	1,646	4	5,095	8,460	3,095	3	9,417	1,428	29,148
% of Total	5.7%	0.01%	17.5%	29.0%	10.6%	0.01%	32.3%	4.9%	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 2020, the total of 4,567 included 1,428 for the above-mentioned combined race and ethnicity categories.

Table 8: CBUs Released for Transplantation for 2016 to 2020[^]

Fiscal Year	NCBI-funded CBU Shipments⁺	Total CBU Shipments*
2016	529	1,154
2017	494	1,050
2018	493	949
2019	459	848
2020	344	702
Total	2,319	4,703

Data Source: NMDP data.

[^] 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.

COVID-19 Impact on the NCBI Program

Partial Stop Work Order

NCBI-contracted banks often work through established partnerships with medical institutions and facilities across the country to collect CBUs. Due to COVID-19-related restrictions implemented nationwide at many medical facilities, several NCBI-contracted banks experienced challenges fulfilling their NCBI contractual requirements to collect a previously agreed upon number of CBUs. In recognition of these limitations, the NCBI program worked with HRSA’s Office of Acquisition Management and Policy to issue a “partial stop work order” (action requires the Contractor to stop any part of the contracted work for a mutually agreed upon time) for the NCBI-contracted banks that needed and wanted them. This pause in contractual activities allowed the banks to reassess their circumstances in light of the adverse impacts of COVID-19.

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 2020, ACBSCT held 20 other meetings.

In 2020, ACBSCT held two virtual meetings, one on April 27 and the second on September 25. During the April meeting, the ACBSCT received several presentations focused on COVID-19 and the early impacts on facilitating blood stem cell transplants, banking CBUs, and collecting additional data on transplant outcomes. Other presentations focused on potential uses of deceased bone marrow in cellular therapy and emergency medicine as well as an overview of the

Patient Access to Cellular Transplant Act and its potential impact on blood stem cell transplantation.

During the September meeting, the Advisory Council received updates on topics related to COVID-19 and its impact on facilitating blood stem cell transplants; trends in blood stem cell transplantation; mismatched marrow transplants; haploidentical transplants; cord blood in regenerative medicine; cord blood derived Chimeric Antigen Receptor Natural Killer Cells; and outcomes of a phase III double unrelated cord blood versus HLA-haploidentical bone marrow study. The ACBSCT approved one recommendation related to cord blood, which reads:

“HRSA should continue to support collection of high-quality cord blood units through the NCBI to support the development of a demonstration project to optimize the utilization of cord blood for transplantation by providing guidance to transplant centers on uses of cord blood and coordinate and share best practices among entities involved in cord blood collection, selection, logistics, and transplant.”

HRSA took this recommendation into consideration and demonstration projects to support collections of high-quality cord blood units have been implemented through the CWBYCTP and NCBI. The outcomes of each demonstration project will be outlined in the next report to Congress. Since being established, the ACBSCT has made 33 recommendations⁸ 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 roster, agendas for upcoming meetings, and meeting summaries are available on the CWBYCTP website.⁹

On November 15, 2010, ACBSCT members recommended that Medicare reimburse for the acquisition of blood, marrow, and cord blood products for hematopoietic transplantation on a cost basis. At the time of this recommendation, acquisition costs associated with allogeneic hematopoietic stem cell transplants were included in the hospital operating costs and the Medicare-Severity Diagnosis Related Group payment for the allogeneic hematopoietic stem cell transplants, when the transplants occurred in the inpatient setting. On December 19, 2020, Section 108 of the Further Consolidated Appropriations Act, 2020 (P.L.116-94) required changes in how Medicare will pay for hematopoietic stem cell transplants, and specified that payment shall be made on a reasonable cost basis for allogeneic hematopoietic stem cell acquisitions that occur in a short-term acute care inpatient hospital. This change became effective for cost reporting periods beginning on or after October 1, 2020 (FY 2021), subsequent to the reporting period covered in this report.

⁸ The ACBSCT recommendations are available at <https://bloodstemcell.hrsa.gov/about/advisory-council/recommendations>.

⁹ Information is available at <http://bloodcell.transplant.hrsa.gov>.

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 unrelated blood stem sources. The CWBYCTP has played a vital role in expanding access to blood stem cell transplants for those from underrepresented racial and ethnic populations and is striving to achieve health equity in its operations. Over 14 years, the CWBYCTP has listed over 23 million volunteer adult bone marrow registrants and 104,500 NCBI CBUs on the donor registry. These programs continue to enhance the lives of thousands of men, women, and children who need potentially life-saving blood stem cell transplants.