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

REPORT TO CONGRESS

Fiscal Year 2022 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) 2022 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 and their activities from October 1, 2021, through September 30, 2022.

The report provides background information about each program, describes their structures and operations, and provides statistics 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 2022. This is an update to the FY 2021 report, which included information through September 30, 2021.

The purpose of the 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.[1] The other 70 percent, or approximately 12,600 people, often search for a matched unrelated adult donor or umbilical CBU through the CWBYCTP. The 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 blood stem cell transplants. Both the CWBYCTP and NCBI enabled thousands of transplant candidates who lacked suitably matched relatives to explore viable options and identify matched unrelated blood stem sources (e.g., bone marrow and cord blood).

The CWBYCTP aims to increase access to blood stem cell transplants for medically 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) by recruiting and increasing the number of donors from such populations to improve their probability of finding a suitable donor. One way the CWBYCTP accomplishes this is by directing contract resources toward patient advocacy, public and professional education, data collection, donor recruitment, and expansion of the size and diversity of the CWBYCTP donor registry. By the end of FY 2022, there were over 24 million volunteer adult bone marrow donor registrants listed through the CWBYCTP. Of those registrants, 4 million (16 percent) self-identified as belonging to an underrepresented racial or ethnic population.

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. NCBI continues to grow and diversify with over 111,000 NCBI CBUs available on the donor registry

[1] Besse, Kelsey, Maiers, Martin, Confer, Dennis, Albrecht, Mark. (2016). On Modeling Human Leukocyte Antigen-Identical Sibling Match Probability for Allogeneic Hematopoietic Cell Transplantation: Estimating the Need for an Unrelated Donor Source. Biol Blood Marrow Transplant. 22. 410-7. 10.1016/j.bbmt.2015.09.012.

through the CWBYCTP. More than 63 percent of the total NCBI CBUs are from underrepresented racial and ethnic populations.

HRSA
Health Resources & Services Administration

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Acronym List

ACBSCT	Advisory Council on Blood Stem Cell Transplantation
ASTCT	American Society for Transplantation and Cellular Therapy
CBB	Cord Blood Bank
CBU	Cord Blood Unit
CED	Coverage with Evidence Development
CIBMTR	Center for International Blood and Marrow Transplant Research
CMS	Centers for Medicare & Medicaid Services
CWBYCTP	C.W. Bill Young Cell Transplantation Program
FY	Fiscal Year
HLA	Human Leukocyte Antigen
HRSA	Health Resources and Services Administration
MDS	Myelodysplastic Syndrome
MUD	Matched Unrelated Donor
NCBI	National Cord Blood Inventory
NMDP	National Marrow Donor Program
OPA	Office of Patient Advocacy
P.L.	Public Law
SCD	Sickle Cell Disease
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 ("Stem Cell Therapeutic and Research Reauthorization Act of 2010"), P.L. 114-104 ("Stem Cell Therapeutic and Research Reauthorization Act of 2015"), and P.L. 117-15 ("Timely ReAuthorization of Necessary Stem-cell Programs Lends Access to Needed Therapies Act of 2021" [TRANSPLANT Act of 2021]), includes a requirement 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 TRANSPLANT Act of 2021 (P.L. 117-15) 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), Health Systems Bureau, Division of Transplantation provides oversight of both the CWBYCTP and NCBI programs (see Figure 1).

The purpose of the 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 blood stem cell transplants to those from underrepresented racial and ethnic populations (American Indian or Alaska Native; Asian; Black or African American; multi-racial; Native Hawaiian or Pacific Islander; Hispanic or Latino). The 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.

The 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 a transplant. The CWBYCTP also offers patient and donor advocacy services, case management services, data collection on transplant outcomes, and educational activities.

The NCBI program contracts with cord blood banks (CBB) to meet the statutory goal of building a public inventory of at least 150,000 new, high-quality, and genetically diverse cord blood units (CBU). 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 (Secretary) and the HRSA Administrator on matters conducted by both the CWBYCTP and the NCBI program.

III. CWBYCTP Overview

The CWBYCTP provides a structure to facilitate blood stem cell transplantation with blood stem cells from unrelated donors for individuals with leukemia and other life-threatening blood, metabolic, or immune system disorders. The CWBYCTP has five functional areas of focus: bone marrow; cord blood; a single point of access to search for and facilitate access to bone marrow and cord blood; patient advocacy; and stem cell transplant outcomes data. Three separate contracts carry out five functions: the Single Point of Access Coordinating Center (SPA-CC), Office of Patient Advocacy (OPA), and the Stem Cell Therapeutic Outcomes Database (SCTOD).[2] Table 1 shows award amounts of appropriated funds under those contracts from 2018 to 2022. The following is a description of the three current contracts:

- The SPA-CC contract is a combination of the Single Point of Access, Bone Marrow Coordinating Center, and Cord Blood Coordinating Center focal areas. 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 bone marrow and cord blood donors, and engages in public and professional educational activities related to blood stem cell donation and transplantation. The SPA-CC also contains a network of CBBs that lists its CBUs and makes them available for transplantation. The SPA-CC maintains a matching and facilitation 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 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 contractor maintains a secure repository for storing donor and recipient samples for research and provides analysis of the clinical outcomes of transplant recipients.

[2] In fiscal year 2022, the Medical College of Wisconsin, the parent organization of the Center for International Blood and Marrow Transplant Research, was the contractor for the SCTOD contract, and the National Marrow Donor Program was the contractor for the OPA and SPA-CC contracts.

Figure 1: CWBYCTP and NCBI Program

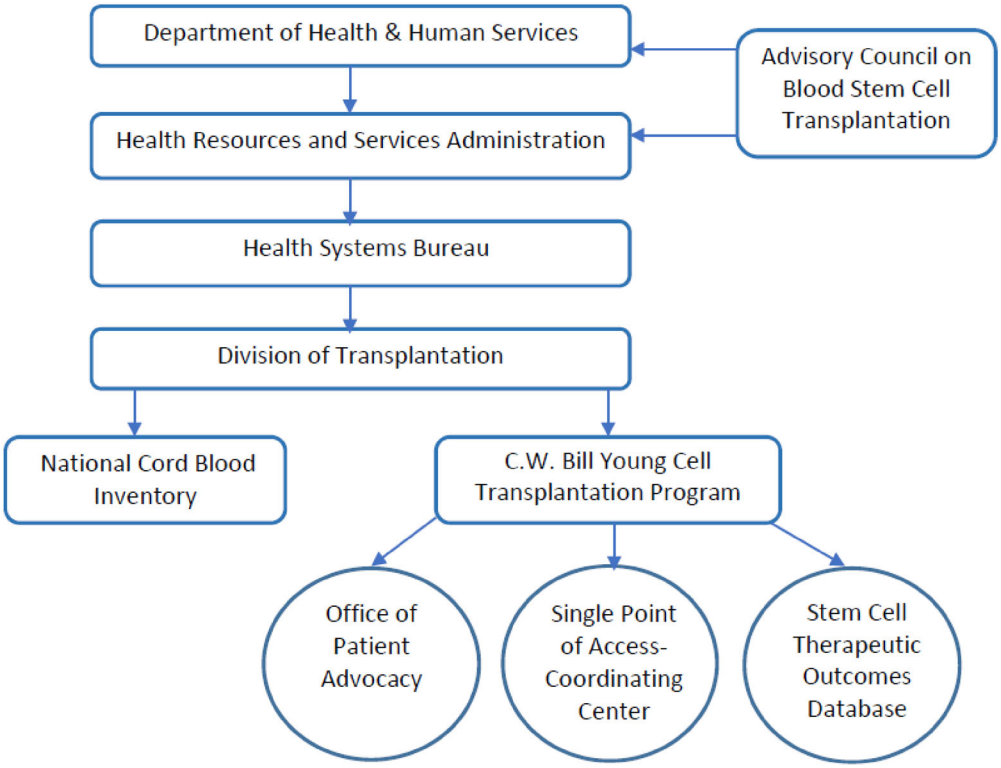

Table 1: Funding for the CWBYCTP Contracts for 2018 to 2022*+

Fiscal Year	Appropriation+	Single Point of Access-Coordinating Center	Office of Patient Advocacy	Stem Cell Therapeutic Outcomes Database	Total Program Contracts
2018	$24,050,000	$17,141,120	$826,934	$4,393,230	$22,361,284
2019	$24,501,000	$16,780,698	$851,741	$4,447,825	$22,080,264
2020	$30,009,000	$21,804,584	$877,293	$4,601,550	$27,283,427
2021>	$31,009,000	$29,888,122	$903,612	$4,730,240	$35,521,974
2022	$32,009,000	$21,343,315	$1,518,782	$5,909,653	$28,771,750
Total	**$141,578,000**	**$106,957,839**	**$4,978,362**	**$24,082,498**	**$136,018,699**

Notes:
*Data Source: HRSA information as of January 6, 2023.
>Fiscal year (FY) 2021 includes $7 million in reprogramming from the NCBI Program to CWBYCTP to support cord blood functions within the legislative authority, of the TRANSPLANT Act of 2021.
+Administrative costs account for differences between appropriations and total program contracts awarded.

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 CBU. The CWBYCTP serves individuals in need of unrelated blood stem cell transplantation and works to ensure that members of medically underrepresented racial and ethnic 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 (MUD) through the CWBYCTP varies by race and ethnicity. For example, the likelihood or probability for Black/African Americans to find a suitable unrelated matched donor is 29 percent compared to 47 percent for Asian/Pacific Islanders, 48 percent for Hispanics/Latinos, 60 percent for American Indians or Alaska Natives, and 79 percent for White, non-Hispanic populations.

As of this report, the total number of volunteer adult bone marrow registrants is over 24 million, with more than 4 million (16 percent) self-identified as belonging to an underrepresented racial or ethnic population. In FY 2022, the CWBYCTP added 250,825 new registrants (Table 2), and 34 percent of those self-identified as belonging to an underrepresented racial or ethnic population, which is more than double the percentage of the overall donor list.

In FY 2022, there were fewer adult donors added to the donor registry than in FY 2021 (Table 2). HRSA attributes this decrease to COVID-19 and the limited number of in-person donor recruitment drives, primarily on college campuses, that were hosted.

Despite the ongoing COVID-19 pandemic, the number of unrelated blood stem cell transplants facilitated by the CWBYCTP increased by eight percentage points from FY 2021 to FY 2022 as shown in Table 3. During the same period, the percentage of transplants facilitated across combined racial and ethnic categories remained relatively flat.

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

Race/Ethnicity	FY 2021	Percentage of Total Number of Registrants Added	FY 2022	Percentage of Total Number of Registrants Added
American Indian or Alaska Native	474	<1%	645	<1%
Asian	13,315	5%	18,952	8%
Black or African American	7,838	3%	13,787	5%
Multi-racial	22,557	9%	21,184	8%
Native Hawaiian or Other Pacific Islander	176	<1%	323	<1%
Unknown+	5,182	2%	14,347	6%
White	156,049	61%	151,170	60%
Hispanic or Latino^	51,080	20%	30,417	12%
Total	**256,671**		**250,825**	

Notes:
*Data Source: National Marrow Donor Program (NMDP) data as of September 30, 2022.
+Unknown reflects people who did not provide race and ethnicity data, which is common for international registries where capturing data by race and ethnicity does not often occur.
^Hispanic or Latino represents a broad ethnicity and donors may be of any race; a separate category is included to prevent individuals from being counted multiple times.

Table 3: Number of Transplants Facilitated by the CWBYCTP by Race/Ethnicity*+

Race/Ethnicity	FY 2021	Percent of Total Number of Transplants Facilitated	FY 2022	Percent of Total Number of Transplants Facilitated
American Indian or Alaska Native	25	0.42%	20	0.31%
Asian	240	4.03%	296	4.61%
Black or African American	288	4.84%	277	4.31%
Multi-racial	7	0.12%	35	0.54%
Native Hawaiian or Other Pacific Islander	10	0.17%	11	0.17%
Unknown^	1,198	20.13%	1,202	18.71%
White	4,178	70.19%	4,574	71.19%
Hispanic or Latino**	6	0.10%	10	0.16%
Total	**5,952**		**6,425**	

Notes:
*Data Source: NMDP data as of September 30, 2022.
+Data in this report may change due to delayed data responses and result in a varying number of transplants reported from prior year reports.
^Unknown reflects people who did not provide race and ethnicity data, which is common for international registries where capturing data by race and ethnicity does not occur.
**Hispanic or Latino represents a broad ethnicity and donors may be of any race; a separate category is included to prevent individuals from being counted multiple times.

Transplant Survival Rates

The CWBYCTP establishes goals for the number of transplants facilitated and for the outcomes of these transplants. To calculate survival rates along with corresponding probability of survival rates, the CWBYCTP contractor uses analytics and reporting tools that provide center-specific survival performance outcome measures for each center.[3] For this FY 2022 report, transplant outcomes for FY 2020 were the latest full dataset available and are reported and compared to FY 2019 data in Table 4. The nature and source of the data cause inherent delays in collecting, reporting, and analyzing data from transplant centers on survival outcomes. Additionally, to report the probability of survival rate at 1 year following transplant, all transplant recipients must be followed for at least a year after the procedure to allow the transplant centers the opportunity for adequate follow-up and reporting. HRSA will report on FY 2021 transplant outcomes data, including the probability of 1-year survival, in the FY 2023 annual report.

As Table 4 reflects, the program performance goal of 69 percent patient survival at 1-year post-transplant was surpassed in all tracked categories in FY 2019 and in all categories except double cord transplant in FY 2020. Double cords are transplants where the recipient is given two units of umbilical cord blood to mitigate for small units and to enhance engraftment. Overall, survival rates remained relatively stable between FY 2019 and FY 2020 across the broad categories for stem cell transplant.

Table 4: 1-year Post-transplant Survival Rate (U.S. Transplant Centers) for 2019 and 2020*

Donor Type	FY 2019	FY 2020
Unrelated Transplants		
Adult Donor	74%	75%
Single Cord	81%	76%
Double Cord	70%	68%
Related Transplants		
Matched Related	80%	82%
Mismatched Related	73%	75%

Notes:
*Data Source: Center for International Blood and Marrow Transplant Research (CIBMTR) data as of December 22, 2022.

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

Centers for Medicare & Medicaid Services Coverage with Evidence Studies

In 2010, 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) to address concerns regarding lack of access to blood stem cell transplant for some individuals diagnosed with Myelodysplastic Syndrome (MDS).

As a result of this request, CMS decided effective August 2010 to provide Coverage with Evidence Development (CED) for blood stem cell transplants for MDS. CMS approved a study that leveraged the reporting requirements of the SCTOD and met the criteria for the CED mechanism. After CMS began providing coverage through Medicare, Medicare beneficiaries with MDS were able to receive blood stem cell transplants in the United States (see Table 5).

Since that decision, CMS has also decided to provide CED for blood stem cell transplants for three other diseases (Myelofibrosis, Multiple Myeloma, and Sickle Cell Disease), along with corresponding CED studies, resulting in a total of four active studies on blood stem cell transplants having been conducted under the 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 2022 are provided in Table 5. However, further information on the latest diseases added will be included in a subsequent report covering FY 2023.

Table 5: Number of Patients Enrolled in CED Studies through September 30, 2022*

Disease Indication	Study Opened	Age	Unrelated Donor	Related Donor	Total FY 2011 - 2022
Myelodysplastic Syndrome	December 2010	65 and older	3,476	1,610	5,086
		Under 65 with Medicare	249	125	374
Myelofibrosis	December 2016	55 and older	316	130	446
Multiple Myeloma	July 2017	Any	13	14	27
Sickle Cell Disease	November 2017	15-50	2	9	11
Total			**4,056**	**1,888**	**5,944**

Notes:
*Data Source: CIBMTR data as of December 22, 2022.

CWBYCTP Professional Education and Outreach Highlights

The COVID-19 pandemic impacted the delivery of educational programs to medical professionals. Prior to COVID-19, many educational programs were delivered in-person and

recorded for online access. From March 2020 through 2021, CWBYCTP conducted all national and regional conferences virtually. In early 2022, in-person professional educational conferences and outreach programs resumed.

Transplant Referral Timing Guidelines and Resources

Timely referral is critical to patients receiving a blood stem cell transplant. The SPA-CC contractor educates those within the blood stem cell community on the importance of transplant referral timing and early human leukocyte antigen (HLA) typing for patients by using the Transplant Consultation Guidelines. These guidelines identify appropriate referral timing for allogeneic or autologous blood stem cell transplants based on a patient's disease characteristics. The SPA-CC contractor developed these guidelines in collaboration with stakeholders in the transplant community based on current clinical practice, medical literature, National Comprehensive Cancer Network® Guidelines for the treatment of cancer, and evidence-based reviews. The SPA-CC contractor updated the guidelines in early 2022 and disseminated them to providers via newsletter in April 2022. The guidelines are available in print, online, and mobile app versions.[4]

In addition to these guidelines, the OPA contractor disseminated peer-reviewed research studies to educate physicians about the impact of the relationship between community hematologists/oncologists and transplant centers in supporting early HLA typing, early referral, and improved patient outcomes.

Health Professional Education Activities and Engagement

In FY 2022, the SPA-CC and OPA contractors offered a variety of programs to educate and engage health care professionals about the need for blood stem cell transplants and services offered through the CWBYCTP. This included programs with continuing education credits for medical professionals; workshops to key stakeholders in the blood stem cell community on transplant issues; disseminating findings from studies on the importance of early HLA typing and referral for optimal patient outcomes; hosting webinars; sponsoring a multi-center Phase II trial; and virtual roundtables for transplant centers to learn about how physicians address challenging patient cases and to share resources that support clinical decision-making. To support health equity and access to blood stem cell transplants, the OPA contractor disseminated several studies via its website and newsletter.[5,6,7]

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

[5] Khera, Nandita, Hahn, Theresa, Brazauskas, Ruta, Jacobs, Benjamin, et. al. (2021). Trends in Use and Outcomes of Autologous and Allogeneic Hematopoietic Cell Transplantation in Racial/Ethnic Minorities. Blood. 138. 427-427. 10.1182/blood-2021-146967.

[6] Horowitz, Mary, Kaur, Manmeet, Mendizabal, Adam, Chen, Min, et. al. (2022). Racial and Ethnic Diversity on Blood and Marrow Transplant Clinical Trials Network (BMT CTN) Trials – We Can Do Better. Transplantation and Cellular Therapy. 28. S71. 10.1016/S2666-6367(22)00244-5.

[7] Strauss, Alexandra, Sidoti, Carolyn, Purnell, Tanjala, Sung, et. al. (2022). Multi-center study of racial, ethnic inequities in liver transplant evaluation: understanding mechanisms, identifying solutions. Liver Transplantation. 28. 10.1002/lt.26532.

The CWBYCTP continues to focus on professional education programs to highlight blood stem cell transplant as a potential cure for sickle cell disease (SCD). Based on feedback from the community, blood stem cell transplant is an underused treatment and the only currently available potential cure for SCD, which primarily affects people of African descent. To reach hematologists and oncologists nationally, the OPA contractor provided content for inclusion in the American Society of Hematology's Sickle Cell Disease Coalition newsletter, a publication reaching thousands of subscribers annually. The CWBYCTP contractor also disseminated SCD resources at the Sickle Cell Community Consortium Annual Leadership Summit (April 2022) and the Foundation for Sickle Cell Disease Research Annual Conference (June 2022) to interact with and educate individuals living with SCD, family members, physicians, nurses, social workers, psychologists, and community health workers.

CWBYCTP Public Educational Resources and Services

Patient Navigation Services and Educational Resources

The SPA-CC and OPA contractors provide patient navigation services and develop resources to guide patients through all phases of transplantation. The SPA-CC and OPA contractors collaborate to provide transplant search data to providers and patients. The OPA contractor's outreach program works with patients to identify concerns and help remove barriers to transplant through referral to services offered by the OPA contractor, the SPA-CC contractor, and other organizations. In FY 2022, the OPA's patient navigators made more than 15,000 outbound calls to patients who have had a search of the registry conducted on their behalf; this number only reflects initial calls made. In addition to phone calls, all potential transplant patients receive information packets in the mail detailing the services and resources available. An analysis of the first 2 years of the program found that patients were 24 percent more likely to progress to transplant if the OPA contractor contacted them through the outreach program.[8]

Post-transplant survival is a primary concern of patients and HRSA. The CWBYCTP contractor provides survival data in two formats: (1) disease-specific survival data through the survival data query tool on the Program website and (2) transplant center-specific survival data through the transplant center directory online.[9,10]

During the period of this report, the OPA contractor continued its focus on outreach and educational resources for people with SCD. Two patient navigators were trained to support the unique needs of this patient population; creating and disseminating SCD outreach packages, which contained fact sheets and other educational materials on available SCD therapies to educate patients. The fact sheets help those living with SCD navigate conversations with health care providers on therapeutic options. Patient navigators sent these packages to more than 90

[8] Preliminary search date October 1, 2019, to September 30, 2021, received donor/cord blood transplant facilitated by OPA contractor within 210 days of preliminary search (April 28, 2022).
[9] Survival Data Query Tool: https://bloodstemcell.hrsa.gov/data/transplant-survival-report.
[10] https://bethematch.org/tcdirectory/search/

individuals. Additionally, the OPA contractor made these resources publicly available through Sickle Cell Connect.[11] During FY 2022, users downloaded these resources nearly 3,900 times from Sickle Cell Connect.

Summary of Published and Unpublished Studies for SCTOD

In FY 2022, the SCTOD contractor developed, conducted, and published research studies in the following relevant areas:

- Evaluating the optimal selection, harvesting, and processing of an unrelated donor graft;
- Evaluating the optimal choice and handling of a CBU for transplantation;
- Comparing alternative donor and graft sources for patients without HLA identical sibling donor(s);
- Evaluating optimal patient selection and treatment strategies for unrelated donor blood stem cell transplant;
- Evaluating optimal patient selection and treatment strategies for cord blood stem cell transplant;
- Evaluating access to care; and
- Evaluating quality of life and late effects of allogeneic blood stem cell transplant.

The SCTOD contractor's completed studies on blood stem cell transplantation resulted in 42 peer-reviewed publications in FY 2022. In addition, there are over 135 studies in progress.[12] A number of these studies are edited into plain language to make them reader-friendly for the public. Also in FY 2022, the SCTOD contractor published 15 plain language or non-scientific summaries, surpassing the SCTOD contractor's annual goal of eight summaries designed specifically for patient use.[13]

SPA-CC Support for Cord Blood Expansion and Adult Donor Recruitment

Demonstration Projects for FY 2022

In FY 2022, HRSA aided transplant teams in cord blood selection, candidate evaluation, and best practices with the goal of increasing cord blood transplants. HRSA also collaborated with ASTCT to educate the workforce on best practices and clinical guidelines to improve cord blood utilization and patient outcomes; supported CBBs in expanding CBU collection in birthing centers; and worked with ASTCT's consultants to support difficult searches.

Highlights from FY 2022 Demonstration Projects

[11] www.sicklecellconnect.com (not supported in Internet Explorer).
[12] The CIBMTR Publication List is available at https://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx.
[13] The Study Summaries for Patients are available at https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/pages/index.aspx.

The Stem Cell Therapeutic and Research Reauthorization Act of 2010, P.L. 111-264 includes a requirement that states, in part:

> *"…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…"*

Below are summaries of four FY 2022 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 and search strategy advice from the CWBYCTP contractor. The goal of this project is to more rapidly identify individuals who are having difficulty finding suitable transplant options to minimize delays in their time to transplant, which is a factor in transplant outcomes. To assist transplant centers in finding a graft source that meets the needs of their patients within their timeframe, search strategy advice provides recommendations for both unrelated donors and CBUs. If cord blood is the best option, cord consultation is available for physicians who have questions about any aspect of the cord blood transplant process. Without this support, physicians with less experience using cord blood might be disinclined to consider cord blood for their patients. In FY 2022, there were two requests for cord consultations, one of which resulted in the patient receiving a double cord transplant using two recommended CBUs.

The CWBYCTP contractor implemented marketing activities to increase awareness across the broader transplant community, as well as transplant centers. Promotional strategies included a digital ads campaign, social media posts, and a blood stem cell transplant Source Selection webinar on cord blood. The CWBYCTP contractor had a booth at its ONE Forum meeting to promote source selection education and developed webpages with donor source selection resources on its Network site.

The CWBYCTP contractor also developed a segmentation plan to reach the transplant centers most likely to benefit from support based on cord blood usage, pediatric designation, and a lower volume of consistent cord blood procurement. This resulted in a list of 23 transplant centers, which received communication on cord blood resources and support.

Provide Support to Enhance Cord Blood Collection Efforts with NCBI Contractors

The CWBYCTP provided support to NCBI contractors Bloodworks, Cleveland Cord Blood Center, Duke University, LifeSouth, and the University of Texas MD Anderson CBB to collect CBUs from underrepresented racial and ethnic populations. This support helped establish and operate new cord blood collection sites, expand collection site hours, and hire and train staff to enhance collections. The CWBYCTP's support also promoted proactive interaction with the

medical community and general public to expand donor diversity, as well as the ability to hire additional collection staff, to allow for 24/7 staffing, and to address burnout and high turnover caused by COVID-19 restrictions. The CBBs collected approximately 2,306 CBUs and added a total of 268 new CBUs to the registry. Of this total, 67 percent were from racially diverse donors.

Plan to Support Engagement with the Cord Blood Community

The cord blood community's priorities have consistently included education and awareness, sustainability of CBB operations and protection of the inventory, and alternative uses of this resource beyond traditional blood stem cell transplant. CBB and transplant center challenges include the imbalance of cord blood supply and demand which threatens the sustainability of CBBs, the declining number of physicians proficient in conducting cord blood transplants, and supply chain and workforce shortages. The CWBYCTP contractor partnered with the cord blood community to strategize on ways to address these challenges and this collaboration resulted in several improvements. The SPA-CC contractor piloted a "Cure-Ready" special project to select CBUs for confirmatory typing and release testing to make CBUs immediately available for patients. Following the success of the pilot, the contractor operationalized the changes across the system, including upgrades and enhancements to simplify cord blood selection. These enhancements include additional plasma and red blood count details for all CBUs, automatic reminders for CBU thresholds established for varying conditions, "Cure-Ready" indicator flags for units that have undergone high-resolution HLA confirmatory typing, and sort and filter features that allow searches to be performed based on cord blood selection criteria inserted by the searching physician. In addition, the contractor established a CBB Alliance, a network of CBBs with a demonstrated history of supplying high-quality CBUs, to increase the use of cord blood in emerging cell and gene therapies.

Support the HLA Today Program

HLA Today is a program that offers early blood stem cell transplant support for community practitioners caring for patients with blood cancers or disorders by providing free HLA typing for patients diagnosed and treated at community oncology/hematology practices. In addition, the program provides community practitioners with a customized search report that includes a summary of potential unrelated donors, related donors, and CBU options to help inform their critical treatment decisions and support the patients' potential journeys to transplant.

In FY 2022, HLA Today produced four webinars, five e-newsletters, four podcast episodes, three print, and 25 online research summaries and updated clinical guidelines on transplant consultation timing.

The Impact of COVID-19 on the CWBYCTP

Throughout FY 2022, HRSA continued to provide support to the SPA-CC and OPA contractors for unanticipated expenses that resulted from the COVID-19 pandemic, including increased

donor recruitment and retention efforts, cryopreservation, and donor and courier travel. Additionally, the CWBYCTP provided support to patients and donors to address COVID-19 concerns. Below are summaries of the support provided.

COVID-19 Resources to Support Patients and Donors

To address patient concerns during the COVID-19 pandemic, the CWBYCTP supported the development of resources to support patients with planned facilitated transplants, including two pre- and post-blood stem cell transplant COVID-19 fact sheets available in English and Spanish. In addition, the OPA contractor held a webinar titled *Coping and Coronavirus* to provide information and answer patient questions about COVID-19.

The COVID-19 pandemic still presents a challenge in engaging young and ethnically diverse donors. Findings from focus group sessions conducted by the SPA-CC contractor revealed that donors have become more individualistic and less likely to engage in volunteer activities that complicate their lives. To overcome this and other barriers, the SPA-CC contractor increased communications to keep donors informed, inspired, and educated on the importance of saying yes once called to donate.

Donors chosen for workup are supported throughout the donation process, from request to collection and recovery. As a part of this process, donors were educated early and often about the importance of sharing any new symptoms of illness both in general and/or consistent with COVID-19, including any known exposure(s) to COVID-19. The SPA-CC contractor worked with the donor to ensure proper care and testing to reduce any impact on the donor and patient.

Support the Processing and Cryopreservation of Blood Stem Cell Products

Given the challenge of international travel during the COVID-19 pandemic, HRSA provided emergency funding to support the processing and cryopreservation of five blood stem cell products to ensure graft availability for transplants. Throughout FY 2022, transplant centers made the decision to proceed with cryopreservation based on the COVID-19 infection rate in their state, their specific hospital policies, and the patient's condition. Beyond the initial support provided by HRSA, the SPA-CC contractor has invested in this activity to allow flexibility in matching donor and patient timelines, and address laboratory capacity issues.

Non-Financial Support of Donor and Courier Travel

HRSA provided travel support for facilitating unrelated blood stem cell transplants for donors and couriers of cord blood products after COVID-19 impacted travel capabilities. Travel was limited if the donor tested positive for COVID-19 or was exposed to someone who did. Couriers encountered challenges in the scheduling and execution of CBU transportation, increased demand on the available cargo systems, and delayed flights. The SPA-CC contractor evaluated and determined methods to reduce the travel distance required and kept collection appointments as much as possible. The SPA-CC contractor, with the assistance of the U.S. Department of

Health and Human Services and HRSA, also engaged non-federal partners to continue offering private pilot aircraft and private corporate flights to transport blood stem cell products when commercial flights were unavailable.

To resolve issues due to the closure of non-preclearance airports in Canada, the U.S. Department of Health and Human Services and HRSA facilitated dispensation by Customs and Border Protection which temporarily permitted the use of preclearance airports to allow onboard couriers to bring products into the United States.

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 also 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 vary by CBB.

HRSA awards contracts to public CBBs through a competitive process and reimburses CBBs on a per CBU basis for each unit that meets all the criteria specified in the contracts. The contracts specify the total number of CBUs HRSA will reimburse per year, and the agreed-upon racial/ethnic mix of donors (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, and the results provide the basis for future funding decisions. When funding is available, HRSA exercises contract options to support the banking of additional CBUs. Table 6 shows the previous 5 years of the NCBI program's appropriations and funding history. FY 2021 funds expended on contract awards were lower than projected due to contracted banks facing challenges meeting collection requirements stemming from the impacts of the COVID-19 pandemic. HRSA reprogrammed $7 million in funding to the CWBYCTP to support activities of the CBBs other than direct CBU purchases.

Table 6: Appropriations and Contract Funding History for the NCBI Program for 2018 to 2022*

FY	Appropriation+	Total Contract Award
2018	$15,236,000	$14,109,672
2019	$16,195,000	$15,194,125
2020	$17,266,000	$16,221,529
2021	$18,266,000	$8,518,294
2022	$18,266,000	$17,301,423
Total	**$85,229,000**	**$71,345,043**

Notes:
*Data Source: HRSA information as of January 6, 2023.
+Administrative costs account for differences between appropriations and total contract awards.

From the inception of the NCBI program in FY 2004 through FY 2022, HRSA awarded 28 NCBI program contracts to 13 different contractors. Figure 2 identifies organizations with contracts as of the end of FY 2022 and their geographic distribution. 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 2022, HRSA contracted with 13 CBBs for NCBI. Those contractors include Carolinas Cord Blood Bank at Duke University, Cleveland Cord Blood Center, CORD: USE Cord Blood Bank, JP McCarthy Cord Stem Cell Bank at Wayne State University, LifeCord Cord Blood Bank at LifeSouth Community Blood Centers, New Jersey Cord Blood Bank at Bergen Community Regional Blood Center, New York Blood Center, Bloodworks, St. Louis Cord Blood Bank at SSM Cardinal Glennon Children's Medical Center, South Texas Blood and Tissue Center (Gencure), StemCyte, Inc., the University of Colorado, and the University of Texas MD Anderson Cancer Center.

NCBI Program Accomplishments and Statistical Highlights

CBBs collected over 25,000 CBUs from FY 2018 through FY 2022 (Table 7), and 51 percent of CBUs shipped through the CWBYCTP were selected from NCBI (Table 8). CBU collection and banking remain key in serving a diverse population. As NCBI's inventory of CBUs grows and becomes more diverse, it will continue to provide increased access to a wider group of patients and enhance health equity. Table 7 provides a breakdown of CBUs contracted by the NCBI program by race and ethnicity over the past 5 years. As noted in Table 7, the number of cord blood units decreased across all racial and ethnic populations. Despite several challenges related to the COVID-19 pandemic, an increase in the cost per cord blood unit, and a decreased number of the program's contractors, the number of CBUs from underrepresented racial and ethnic populations continues to account for 61 percent of units collected through the program.

As shown in Table 8, the number of non-NCBI CBUs released for transplant has decreased since FY 2018, primarily due to the increased use of alternative therapies, including haploidentical transplants. Haploidentical transplants use blood stem cells from donors who are biologically related to the recipient and are not facilitated through the CWBYCTP. Despite this decrease in non-NCBI cord blood transplants, patient access to potentially life-saving blood stem cell treatments across racial and ethnic categories from NCBI units and facilitated by the CWBYCTP remained relatively constant (Tables 3 and 8).

Table 7: Contracted NCBI CBUs by Race/Ethnicity for 2018 to 2022*

FY	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
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
2021	185	—	482	1,072	—	—	1,621	757	4,117
2022	256	—	591	1331	—	—	1,364	893	4,435
Total	**1,368**	**2**	**3,947**	**7,624**	**1,196**	**0**	**8,276**	**3,078**	**25,491**
% of Total	**5%**	**0%**	**15%**	**30%**	**5%**	**0%**	**32%**	**12%**	**100%**

Notes:
*Data Source: Internal HRSA information as of January 31, 2023.
+American Indian or Alaska Native.
^Black or African American.
**Hispanic or Latino represents a broad ethnicity and donors may be of any race; a separate category is included to prevent individuals from being counted multiple times.
#Native Hawaiian or other Pacific Islander.
Percent 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.

Table 8: CBUs Released for Transplantation from 2018 to 2022*

FY	NCBI-funded CBU Shipments	Total CBU Shipments[+]
2018	493	949
2019	459	848
2020	344	702
2021	313	589
2022	342	576
Total	**2,103**	**4,138**

Notes:
*Data Source: NMDP data as of February 23, 2023
[+]Includes both NCBI and non-NCBI CBUs.

V. Advisory Council on Blood Stem Cell Transplantation

Per the Stem Cell Therapeutic and Research Act of 2005 (P.L. 109-129), as amended, the Secretary established the ACBSCT to advise the Secretary and the HRSA Administrator on matters related to the CWBYCTP and the NCBI program. The ACBSCT held 20 meetings between January 2008 and September 2020. In FY 2022, HRSA onboarded new members to the ACBSCT, and will meet in FY 2023 to review issues related to the CWBYCTP and the NCBI program.

Summary

Despite ongoing challenges related to COVID-19, the CWBYCTP, and NCBI worked to expand access to blood stem cell transplants for all patients, including those from underrepresented racial and ethnic populations, and carried out initiatives to achieve health equity in its operations. Although the effects of COVID-19 adversely impacted the ability to host live drives in some areas in FY 2022, the CWBYCTP added over 250,000 adult donor registrants and facilitated more transplants with mismatched unrelated transplants than the prior year. The CWBYCTP facilitated more than 6,400 transplants overall, with approximately 1,850 of those transplants (or 28.8 percent) for underrepresented racial and ethnic populations.

In addition, the OPA, SCTOD, and SPA-CC contractors continued to work together to provide public and professional education and engage with stakeholders in the blood stem cell community to advance research and improve transplant outcomes.

The NCBI program continued to play a vital role in providing transplant candidates with an additional blood stem cell source by adding thousands of CBUs to its inventory.

With the sustained support of Congress, these programs will continue to save and enhance the lives of thousands of men, women, and children who need a potentially life-saving blood stem cell transplant.