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

REPORT TO CONGRESS

Fiscal Year 2023 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) 2023 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, 2022, through September 30, 2023.

The report provides background information about each program, describes each structure and operation, and provides statistics on the number of bone marrow donor registrants and collected cord blood units (CBU), along with other data. Unless otherwise noted, the information presented is from FY 2023. This is an update to the FY 2022 report, which included information through September 30, 2022.

The purpose of CWBYCTP is to increase the number of bone marrow and cord blood transplants for recipients matched to biologically unrelated donors.[1] Every year, approximately 18,500 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 25 percent of people have a fully matched relative.[2] The other 75 percent, or nearly 14,000 people, will often require a search for a matched unrelated adult donor or umbilical CBU through CWBYCTP. CWBYCTP supports the infrastructure for identifying, matching, and facilitating the distribution of bone marrow, peripheral blood stem cells, and cord blood from unrelated donors for individuals in need of blood stem cell transplants. Both 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, peripheral blood stem cells, and cord blood).

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 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 2023, there were over 41 million volunteer adult bone marrow donor registrants accessible through the CWBYTCTP, of which 9.5 million registrants are within the United States. Of the U.S. registrants, 3.8 million (40 percent) self-identified as belonging to an underrepresented racial or ethnic population.

The Health Resources and Services Administration, through its NCBI program, contracts with cord blood banks to purchase CBUs to help meet the goal of building a public inventory of at

[1] Blood stem cell transplants include transplants using bone marrow, cord blood, or peripheral blood stem cells.

[2] 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. Doi: 22. 410-7.10.1016/j.bbmt.2015.09.012.

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 122,000 NCBI CBUs available on the donor registry through CWBYCTP. More than 62 percent of the total NCBI CBUs are from underrepresented racial and ethnic populations.

CWBYCTP continued to serve a diverse patient population, with volunteer adult donors and CBUs playing a vital role in expanding transplant access to all patients, including underrepresented racial and ethnic populations. CWBYCTP facilitated nearly 7,000 transplants overall, with approximately 1,500 of those transplants (or 21 percent) for underrepresented racial and ethnic populations.

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Acronym List

ACBSCT	Advisory Council on Blood Stem Cell Transplantation
CBB	Cord Blood Bank
CBU	Cord Blood Unit
CWBYCTP	C.W. Bill Young Cell Transplantation Program
FY	Fiscal Year
HCT	Hematopoietic Cell Transplantation
HLA	Human Leukocyte Antigen
HRSA	Health Resources and Services Administration
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, and 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 CWBYCTP and NCBI programs (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 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). 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 a transplant. 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 CWBYCTP.

The role of ACBSCT is to advise, assist, consult with, and make recommendations to the Secretary of Health and Human Services (Secretary), acting through the HRSA Administrator, on matters conducted by both CWBYCTP and the NCBI program.

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

CWBYCTP provides a structure to facilitate blood stem cell transplantation with blood stem cells from unrelated donors (marrow and cord blood) for individuals with leukemia and other life-threatening blood, metabolic, or immune system disorders. 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), the Office of Patient Advocacy (OPA), and the Stem Cell Therapeutic Outcomes Database (SCTOD). In fiscal year (FY) 2023, 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 (NMDP) was the contractor for OPA and SPA-CC contracts. Table 1 shows award amounts of appropriated funds under those contracts from 2019 to 2023. 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. 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 blood stem cell 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. SPA-CC also contains a network of CBBs that lists its CBUs and makes them available for transplantation. 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. OPA provides public and professional education, information, resources, and support for blood stem cell 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.

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

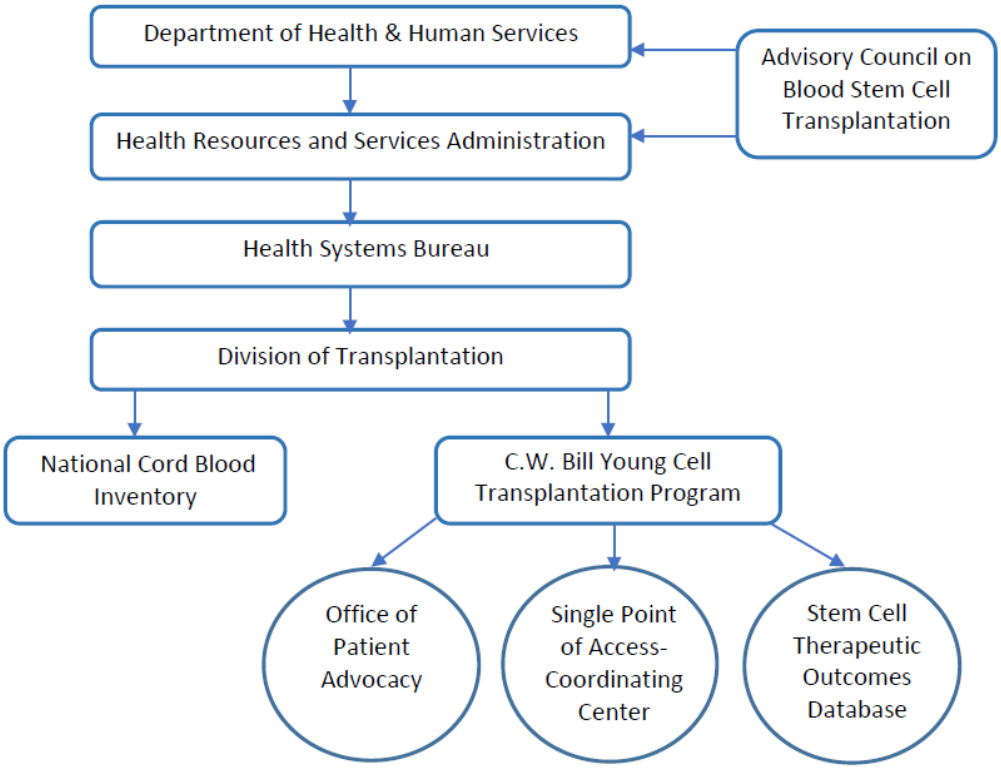

Table 1: Funding for the C.W. Bill Young Cell Transplantation Program Contracts for 2019 to 2023*

Fiscal Year	Appropriation+	Single Point of Access - Coordinating Center	Office of Patient Advocacy	Stem Cell Therapeutic Outcomes Database	Total Program Contracts
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
2023	$33,009,000	$23,364,246	$1,564,345	$5,727,767	$30,656,358
Total	$150,537,000	$113,180,965	$5,715,773	$25,417,035	$144,313,773

Notes:
*Data Source: HRSA accessed on January 6, 2023.
> 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.

C.W. Bill Young Cell Transplantation Program Statistical Updates

Every year, approximately 18,500 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 25 percent of people have a fully matched relative. The other 75 percent, or nearly 14,000 people, often search for a matched unrelated adult donor or umbilical CBU. 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 through CWBYCTP varies by race and ethnicity. Recent advancements in transplantation procedures are expanding the definition of suitable donors to include both fully matched and partially mismatched donors. Overall, these advances increase the likelihood of finding a suitably matched donor for all patients in need of a transplant. For example, the likelihood or probability for Black or African Americans to find a fully matched (8/8 human leukocyte antigen (HLA)) unrelated donor is 29 percent compared to 50 percent for Asian/Pacific Islanders, 48 percent for Hispanics/Latinos, 58 percent for American Indians or Alaska Natives, and 80 percent for White-non-Hispanic populations. With the advances in transplantation that enable favorable outcomes using partially mismatched donors, the likelihood or probability for Black or African Americans to find a suitable unrelated donor increases to 84 percent compared to 92 percent for Asian/Pacific Islanders, 90 percent for Hispanics/Latinos, 93 percent for American Indians/Alaska Natives, and 98 percent for White-non-Hispanic populations.[3]

As of this report, there were more than 41 million volunteer adult bone marrow donor registrants accessible through CWBYCTP, of which 9.5 million registrants are within the United States. Of the U.S. registrants, 3.8 million (40 percent) self-identified as belonging to an underrepresented racial or ethnic population. In FY 2023, CWBYCTP added 339,489 new registrants (Table 2), and 36 percent of those self-identified as belonging to an underrepresented racial or ethnic population, which is more than the 34 percent who self-identified in FY 2022. In FY 2023, more adult donors were added to the donor registry than in FY 2022 (Table 2).

In FY 2023, the total number of unrelated blood stem cell transplants facilitated by CWBYCTP increased by 8 percentage points from more than 6,400 in FY 2022 to nearly 7,000 in FY 2023, an increase of approximately 450. During the same period, the percentage of transplants facilitated across combined racial and ethnic categories increased by 124 percent (excluding patients who are White-non-Hispanic or unknown race or ethnicity), from almost 650 in FY 2022 to more than 1,450 in FY 2023. The number of transplants for each race or ethnic group is shown in Table 3.

The SPA-CC contractor continued to make progress in increasing both the number of ethnically diverse potential donors on the registry and the number of transplants performed for ethnically

[3] Chowdhury, A.S., Maiers, M., Spellman, S.R., Deshpande, T., Bolon Y.T., Devine, S.M. Existence of HLA-Mismatched Unrelated Donors Closes the Gap in Donor Availability Regardless of Recipient Ancestry. *Transplantation and Cellular Therapy*. 2023 Nov; 29(11): 686.e1-686.e8. doi: 10.1016/j.jtct.2023.08.014. Epub 2023 Aug 14. PMID: 37586457.

diverse patients due to increased focus on ethnically diverse communities, optimizing media and marketing activities to reach these communities more effectively, and educational efforts to build trust within these communities.

Table 2: Number of Registrants Added to the C.W. Bill Young Cell Transplantation Program by Race/Ethnicity*

Race/Ethnicity	FY 2022	Percentage of Total Number of Registrants Added	FY 2023	Percentage of Total Number of Registrants Added
American Indian or Alaska Native	645	<1%	650	<1%
Asian	18,952	8%	29, 883	9%
Black or African American	13,787	6%**	22,471	7%
Multi-racial	21,184	8%	26, 937	8%
Native Hawaiian or Other Pacific Islander	323	<1%	463	<1%
Unknown+	14,347	6%	45,481	13%
White-non-Hispanic	151,170	60%	173,481	51%
Hispanic or Latino^	30,417	12%	40,123	12%
Total	250,825		339,489	

Notes:

*Data Source: NMDP data as of September 30, 2023. Data is subject to change.

**This percentage is 5.5 percent, which we rounded to 6 percent. Data being rounded can result in totals above 100 percent.

+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 C.W. Bill Young Cell Transplantation Program by Race/Ethnicity*

Race/Ethnicity	FY 2022	Percent of Total Number of Transplants Facilitated	FY 2023	Percent of Total Number of Transplants Facilitated
American Indian or Alaska Native	20	<1%	28	<1%
Asian	296	5%	269	4%
Black or African American	277	4%	345	5%
Multi-racial	35	<1%	117	2%
Native Hawaiian or Other Pacific Islander	11	<1%	9	<1%
Unknown^	1,202	19%	675	10%
White-non-Hispanic	4,574	71%	4,832	69%
Hispanic or Latino**	10	<1%	689	10%
Total	6,425		6,964	

Notes:
*Data Source: NMDP data as of September 30, 2023.
+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. This is common when working with 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

CWBYCTP establishes goals for the number of transplants facilitated and for the outcomes of these transplants. To calculate survival rates along with the corresponding probability of survival rates, the CWBYCTP contractor uses analytics and reporting tools that provide center-specific survival performance outcome measures for each center.[4] For this FY 2023 report, transplant outcomes for FY 2021 were the latest full dataset available and are reported and compared to FY 2020 data in Table 4. Additionally, to report the probability of survival rate at 1-year following transplant, all transplant recipients must be followed for at least 1-year after the procedure to allow the transplant centers the opportunity for adequate follow-up and reporting. HRSA will report on FY 2022 transplant outcomes data, including the probability of 1-year survival, in the FY 2024 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 except double cord transplants in FY 2020. However, the outcomes for double cord blood transplants increased by 8 percentage points, from FY 2020 to FY 2021. Double cords are transplants in which the recipient is given two units of umbilical cord blood to mitigate for small units and to enhance engraftment. Overall, survival

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

rates remained relatively stable between FY 2020 and FY 2021 across the broad categories for stem cell transplant.

Table 4: 1-year Post-transplant Survival Rate (U.S. Transplant Centers) for 2020 and 2021*

Donor Type	FY 2020	FY 2021
Unrelated Transplants		
Adult Donor	75%	74%
Single Cord	76%	80%
Double Cord	68%	76%
Related Transplants		
Matched Related	82%	80%
Mismatched Related	75%	75%

Notes:
*Data Source: Center for International Blood and Marrow Transplant Research data as of November 22, 2023.

C.W. Bill Young Cell Transplantation Program Professional Education and Outreach Highlights

Throughout FY 2023, the OPA contractor provided educational opportunities to health care providers in hematology and blood stem cell transplantation. Advances in transplantation have contributed to increased donor options for patients and improved outcomes; therefore, the contractor organized educational efforts around these advances to encourage timely referral and the use of evidence-based guidelines when considering referral for a transplant consult.

Transplant Referral Timing Resources to Improve Access and Outcomes

Timely referral is critical to patients accessing a blood stem cell transplant and achieving optimal outcomes. The OPA contractor educates those within the blood stem cell community on the latest research and advances affecting outcomes and access to transplants, as well as the importance of timely transplant referral and early 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 OPA 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 OPA contractor disseminated the guidelines throughout FY 2023, including highlights in newsletters to transplant providers in February and September 2023. The

guidelines are available in print, online, and mobile app versions.[5] To make the guidelines even more accessible to transplant providers, the OPA contractor substantially updated the mobile app in FY 2023 to provide pre- and post-transplant guidelines and additional educational content. In addition to the guidelines, the OPA contractor disseminates 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 2023, SPA-CC and OPA contractors offered a variety of programs to educate and engage healthcare professionals about the need for blood stem cell transplants and services offered through 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; and hosting webinars and virtual roundtables for transplant centers to learn about how physicians address challenging patient cases and to share resources that support clinical decision-making.

Education focused on expanding access to transplants, optimizing donor selection, and improving outcomes. The OPA contractor, in partnership with the American Society for Transplantation and Cellular Therapy, developed a six-part online education program, "The Evolution of hematopoietic cell transplantation (HCT) [sic] Self-Paced Learning Course."[6] The series covered transplant basics and outcomes, patient eligibility for transplant, donor availability, addressing barriers, shared care practices, and preparing patients for successful HCT. The OPA contractor delivered educational content on these topics through online education modules, webinars, e-newsletters, podcasts, and published research summaries.

CWBYCTP continues to focus on professional education programs to highlight blood stem cell transplant as a curative option for many diseases, including Acute Myeloid Leukemia, high-risk Myelodysplastic Syndrome, and Sickle Cell Disease (SCD). This includes emphasizing advances in precision medicine and measurable residual disease testing to predict and prevent Acute Myeloid Leukemia relapse and advances in risk stratification criteria that expand the use of transplants for Myelodysplastic Syndrome.

During the period of this report, the OPA contractor continued its focus on outreach and educational resources for people with SCD. Patient navigators continue to focus on supporting this patient population's unique needs. The contractors disseminated SCD outreach packages which contain educational handouts regarding SCD therapies and resources available through OPA.

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[5] NMDP. The Transplant Consultation Timing Guidelines. Available at https://bethematchclinical.org/transplant-indications-and-outcomes/referral-timing-guidelines/.

[6] NMDP. The Evolution of HCT Self-Paced Learning Course. Available at https://bethematchclinical.org/medical-education-and-research/education-catalog/the-evolution-of-hct-self-paced-learning-course/.

C.W. Bill Young Cell Transplantation Program Patient Services and Public Educational Resources

Patient Navigation Services and Educational Resources

SPA-CC and OPA contractors provide patient navigation services and develop resources to guide patients through all phases of transplantation. SPA-CC and OPA contractors collaborate to provide transplant search data to providers and patients. 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 2023, OPA's patient navigators made more than 18,000 outbound calls to patients who have had a search of the registry. 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.[7]

Post-transplant survival is a primary interest 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.[8,9]

Summary of Published and Unpublished Studies for Stem Cell Therapeutic Outcomes Database

In FY 2023, the SCTOD contractor developed, conducted, and published research studies in the following 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.

SCTOD contractor's completed studies on blood stem cell transplantation resulted in 48 peer-reviewed publications in FY 2023.[10] In addition, there are nearly 190 studies in progress.[11] A

[7] 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).
[8] HRSA. Transplant Survival Rates Survival Data Query Tool. Available at https://bloodstemcell.hrsa.gov/data/transplant-survival-report.
[9] NMPD. Transplant Center Search. Available at https://bethematch.org/tcdirNectory/search/.
[10] CIBMTR. Publications. Available at https://cibmtr.org/CIBMTR/Resources/Publications.
[11] CIBMTR. All Studies. Available at https://cibmtr.org/CIBMTR/Studies/All-Studies.

number of these studies are edited into plain language to make them reader-friendly for the public. During FY 2023, the SCTOD contractor also published 15 plain language or non-scientific summaries, surpassing the SCTOD contractor's annual goal of eight summaries designed specifically for patient use.[12]

Single Point of Access - Coordinating Center Support for Cord Blood Expansion and Adult Donor Recruitment

Demonstration Projects for FY 2023

In FY 2023, HRSA supported CBBs in expanding CBU collection in birthing centers and in increasing the number of CBUs that have had high-resolution typing performed on them. HRSA also supported a plan for the cord blood community to create an immersion program in cord blood transplantation for junior clinicians to gain hands-on experience with expert mentors.

Highlights from FY 2023 Demonstration Projects

The Stem Cell Therapeutic and Research ReAuthorization Act of 2015, P.L. 111-264, as amended, 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 [sic] receiving a contract under the National Cord Blood Inventory program…"*

Below is a summary of the FY 2023 project.

Provide Support to Enhance Cord Blood Collection Efforts with NCBI Contractors

CWBYCTP provided support to NCBI contractors Bloodworks, Cleveland Cord Blood Center, Duke University, LifeSouth, and the University of Texas MD Anderson CBB to sustain the collection of CBUs from within underrepresented racial and ethnic populations. This support helped establish and operate new cord blood collection sites, expand collection site hours, support recruitment initiatives, and hire and train staff to enhance collections. CWBYCTP's support also promoted proactive interaction with the medical community and the general public to expand donor diversity. Additionally, CWBYCTP supported the ability to hire additional collection staff, to allow for 24/7 staffing and to address burnout and high turnover. The CBBs collected more than 2,000 CBUs and added nearly 250 new CBUs to the registry. Of this total, 71 percent were from ethnically diverse donors.

[12] CIBMTR. Study Summaries. Available at https://cibmtr.org/CIBMTR/Utility-Nav/Patients/Study-Summaries.

IV. National Cord Blood Inventory Program Overview

The NCBI program contracts with CBBs to meet the statutory goal of building a public inventory of at least 150,000 new, high-quality, genetically diverse CBUs, available to individuals through the CWBYCTP donor registry. CBBs may 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 6). 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 5 shows the previous 5 years of 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 CWBYCTP to support activities of the CBBs other than direct CBU purchases.

Table 5: Appropriations and Contract Funding History for the National Cord Blood Inventory Program for 2019 to 2023*

FY	Appropriation+	Total Contract Award
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	$17,391,017
2023	$19,266,000	$18,391,615
Total	$89,259,000.00	$75,588,148

Notes:
*Data Source: HRSA information as of January 3, 2023.
+Administrative costs account for differences between appropriations and total contract awards.
^ In 2021, HRSA reallocated $7.0 million from the National Cord Blood Inventory (NCBI) Program to C.W. Bill Young Cell Transplantation to support cord blood functions through Demonstration Projects. The Demonstration Projects aimed to increase cord blood utilization and improve transplant outcomes.

From the inception of the NCBI program in FY 2004 through FY 2023, HRSA awarded 28 NCBI program contracts to 13 different contractors. Figure 2 identifies organizations with contracts as of the end of FY 2023 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: National Cord Blood Inventory Banks*

*Data Source: Figure created by HRSA with publicly available information. As of the end of FY 2023, HRSA contracted with 10 CBBs for NCBI CBUs. Those contractors include five CBBs still operating active collections: Carolinas Cord Blood Bank at Duke University, Cleveland Cord Blood Center, LifeCord Cord Blood Bank at LifeSouth Community Blood Centers, Bloodworks, and the University of Texas MD Anderson Cancer Center, and five CBBs with maintenance contracts for units previously collected: JP McCarthy Cord Stem Cell Bank at Wayne State University, New Jersey Cord Blood Bank at Bergen Community Regional Blood Center, New York Blood Center, St. Louis Cord Blood Bank at SSM Cardinal Glennon Children's Medical Center, and South Texas Blood and Tissue Center (Gencure).

National Cord Blood Inventory Program Accomplishments and Statistical Highlights

CBBs collected over 21,000 CBUs from FY 2019 through FY 2023 (Table 6) and 54 percent of CBUs shipped through CWBYCTP were selected from NCBI (Table 7). 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 6 provides a breakdown of CBUs contracted by the NCBI program by race and ethnicity over the past 5 years. CBUs from underrepresented racial and ethnic populations continue to account for 61 percent of units collected through the program. Lower collection totals in FYs 2021 through 2023 reflect the residual impact of the COVID-19

pandemic, as well as the staffing shortages that occurred in many collection facilities, and the increased cost to collect, test, and maintain or store CBUs long-term. Restrictions related to the Zika virus have also impacted cord blood collections significantly due to the lack of a Food and Drug Administration approved test for the Zika virus. Currently, if a donor mother has traveled to a known area that is listed on a map for Zika exposure, the mother cannot consent to donate a CBU. This challenge has especially impacted the collection centers in the southern United States, with CBBs reporting up to 30 percent of potential donor mothers being deferred due to Zika risk.

As shown in Table 6, the number of non-NCBI CBUs released for transplant has decreased since FY 2019, 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 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 CWBYCTP remained relatively constant (Tables 3 and 7).

Table 6: Contracted National Cord Blood Inventory Cord Blood Units by Race/Ethnicity for 2019 to 2023*

FY	Asian	Black or African American	Hispanic or Latino**	White	Multi-race, AI/AN, NH/PI+ (2019)	Totals
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	1,331	1,364	893	4,435
2023	420	347	1,217	1,366	825	4,175
Total	**1,418**	**2,879**	**6,651**	**7,028**	**3,903**	**21,879**
% of Total	**6%**	**13%**	**30%**	**32%**	**18%**	**99%***

Notes:
*Data Source: Internal HRSA information as of September 30, 2023.
**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.
+FY 2019 contracted NCBI CBUs combined three race and ethnicity categories, multi-racial, American Indian or Alaska Native (AI/AN), and Native Hawaiian or other Pacific Islander (NH/PI), that were separated in previous NCBI-contracted CBUs.
***Totals do not equal 100 percent due to rounding.

Table 7: Cord Blood Units Released for Transplantation from 2019 to 2023*

FY	NCBI-funded CBU Shipments	Total CBU Shipments[+]
2019	459	848
2020	344	702
2021	313	589
2022	342	576
2023	281	506
Total	**1,739**	**3,221**

Notes:
*Data Source: NMDP data as of September 30, 2023.
[+] Includes both NCBI and non-NCBI CBUs.

Support to Increase Cord Blood Utilization

Since 2007, HRSA has provided funds to CBBs to collect and store high-quality CBBs; however, there has not yet been a consistent effort to increase access to and utilization of these potentially life-saving products for patients. HRSA is increasing its collaboration with the cord blood community, including CBBs, to identify projects to support ways to increase utilization of high-quality CBUs and ways to expand access to cord blood collections at new or existing collection sites.

Plan to Collaborate with the Cord Blood Community

Cord blood addresses some patients' need for access to diverse HLA-typed products. These products are readily available within CBBs, safe for patient use, and offer similar outcomes. However, the use of cord blood for blood stem cell transplant, though an effective therapy, has steadily declined since its peak in 2011 and the practice of cord blood transplantation has become increasingly concentrated among a small number of transplant centers. Therefore, in FY 2023, HRSA recognized the importance of offering technical assistance to the field and generating interest in cord blood transplantation from the next generation of physicians. HRSA is working with those in the cord blood field, including NCBI contractors, to identify projects to increase cord blood utilization of high-quality CBUs. These efforts may increase access to cord blood transplantation, as supported by the ACBSCT (as discussed in Section V).

Perform High-Resolution Human Leukocyte Antigen Typing of National Cord Blood Inventory Cord Blood Units

One of the benefits of cord blood as a stem cell source is rapid availability and the short timeframe between being ordered by a transplant center and shipped from the CBB. An effective way to make the delivery of these units even faster, improve search quality, and enhance matching is through the completion of high-resolution HLA typing and listing in CWBYCTP. To increase the number of CBUs that have had high-resolution typing performed, two NCBI contractors (Cleveland Cord Blood Center and LifeSouth) performed high-resolution HLA typing on approximately 575 CBUs. The CBBs were also able to perform recruitment typing on

nearly 300 CBUs, confirmatory typing on almost 350 CBUs, and release testing on more than 85 CBUs. The HLA typing program met the goal of making CBUs more rapidly available to patients.[13]

V. Advisory Council on Blood Stem Cell Transplantation

Per the Stem Cell Therapeutic and Research Act of 2005 (P.L. 109-129), and 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]), the Secretary established ACBSCT to advise the Secretary and the HRSA Administrator on matters related to CWBYCTP and the NCBI program. In FY 2022, HRSA onboarded new members to ACBSCT, and the council met three times in FY 2023 (i.e., December 5, 2022, December 6, 2022, and September 28, 2023) to review issues related to CWBYCTP and the NCBI program. In December 2022, ACBSCT decided to establish two subcommittees to further discuss strategies to increase cord blood utilization and strategies to address shortages of drugs used to treat individuals who have received a blood stem cell transplant.

Summary

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.

CWBYCTP added approximately 298,000 adult donor registrants and facilitated more transplants than in FY 2022, with mismatched unrelated transplants contributing to the increase. CWBYCTP facilitated nearly 7,000 transplants overall, with approximately 1,500 of those transplants (or 21 percent) performed on individuals from underrepresented racial and ethnic populations.

In addition, OPA, SCTOD, and SPA-CC contractors continued to work together to provide public and professional educational opportunities 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 adults and children who need a potentially life-saving blood stem cell transplant.

[13] Data Source: NMDP data as of September 30, 2023.