



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

REPORT TO CONGRESS

**Fiscal Year 2021 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) 2021 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, 2020, through September 30, 2021.

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 (CBUs), along with other data. Unless otherwise noted, the information presented is from FY 2021. This is an update to the FY 2020 report, which included information through September 30, 2020.

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. 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 hematopoietic stem cell transplants. Both the CWBYCTP and NCBI 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.).

The CWBYCTP aims to increase access to hematopoietic stem cell transplants (HSCT) by 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 in order 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 2021, 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.

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 116,000 NCBI CBUs available on the donor registry through the CWBYCTP. More than 63 percent of the total NCBI CBUs are from underrepresented racial and ethnic populations.



Fiscal Year 2021 Annual Progress Report on the C.W. Bill Young Cell Transplantation Program and National Cord Blood Inventory Program

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Acronym List

ACBSCT	Advisory Council on Blood Stem Cell Transplantation
ASTCT	American Society for Transplantation and Cellular Therapy
CBB	Cord Blood Bank
CBU	Cord Blood Unit
CED	Coverage with Evidence Development
CE	Continuing Education
CIBMTR	Center for International Blood and Marrow Transplant Research
CMS	Centers for Medicare & Medicaid Services
CMV	Cytomegalovirus
COVID-19	Coronavirus Disease 2019
CWBYCTP	C.W. Bill Young Cell Transplantation Program
FY	Fiscal Year
HLA	Human Leukocyte Antigen
HRSA	Health Resources and Services Administration
HSCT	Hematopoietic Stem Cell Transplants
MDS	Myelodysplastic Syndrome
NCBI	National Cord Blood Inventory
NMDP	National Marrow Donor Program
OPA	Office of Patient Advocacy
P.L.	Public Law
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, P.L. 114-104, 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 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 program (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 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 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 an HSCT. 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.

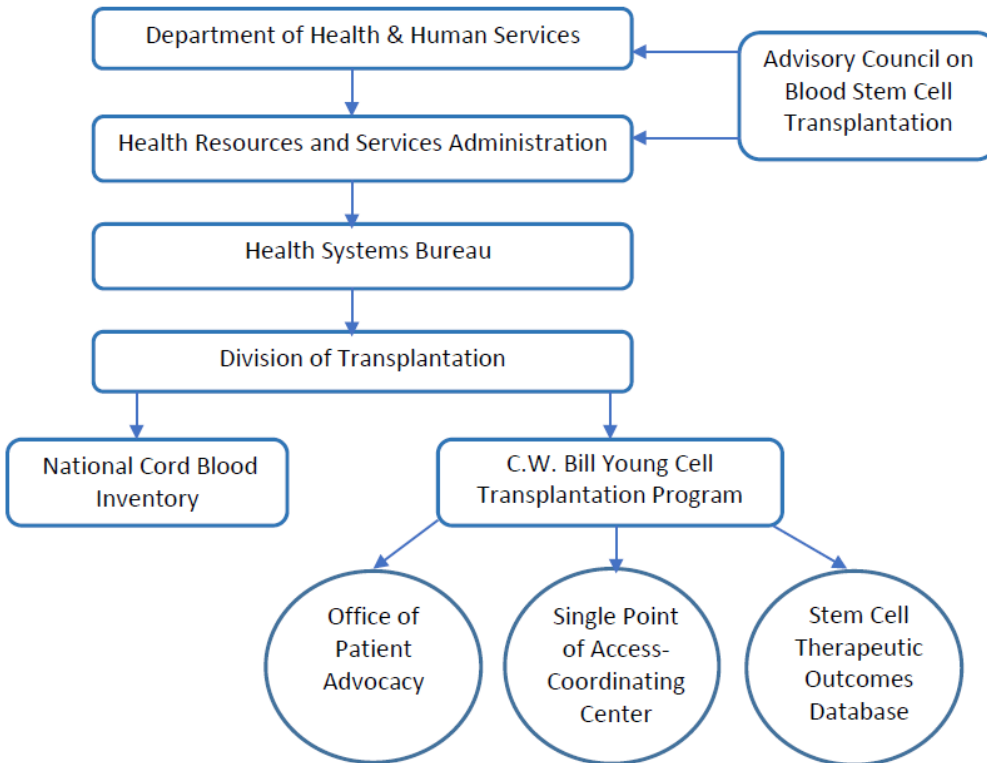
III. CWBYCTP Overview

The CWBYCTP provides a structure to facilitate blood stem cell transplantation with blood-forming cells from unrelated donors for individuals with leukemia and other life-threatening blood, metabolic, or immune system disorders. The CWBYCTP has five 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. Four separate contracts originally carried out the five focal areas: the Bone Marrow Coordinating Center contract, the Cord Blood Coordinating Center contract, the Single Point of Access, Office of Patient Advocacy (OPA) contract, and the Stem Cell Therapeutic Outcomes Database (SCTOD) contract.¹ In 2017, HRSA combined some of the focal areas to form three contracts. Table 1 shows award amounts of appropriated funds under those contracts from 2017 to 2021. The following is a description of the three current contracts:

- The Single Point of Access-Coordinating Center (SPA-CC) contract is a combination of the SPA, 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 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 contract 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 2021, 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



Data Source: Internal HRSA information.

Table 1: Funding for the CWBYCTP Contracts for 2017 to 2021*

Fiscal Year	Appropriation ⁺	Single Point of Access-Coordinating Center	Office of Patient Advocacy	Stem Cell Therapeutic Outcomes Database	Total Program Contracts
2017	\$22,056,000	\$14,640,000	\$802,849	\$4,305,380	\$19,748,229
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
Total	\$131,625,000	\$100,254,524	\$4,262,429	\$22,478,225	\$126,995,178

Notes:

*Data Source: Internal HRSA financial information as of February 23, 2022.

+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. 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. 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 through the CWBYCTP varies by race and ethnicity. For example, the chance of finding a suitably matched unrelated donor varies from 29 percent for Black/African American; 47 percent for Asian/Pacific Islander; 48 percent for Hispanic/Latino; 60 percent for American Indian or Alaska Native; to 79 percent for White, non-Hispanic populations.

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. The CWBYCTP added 256,671 new registrants in fiscal year (FY) 2021 (see Table 2) and 37 percent of those self-identified as belonging to an underrepresented racial or ethnic population.

In FY 2021, there were fewer adult donors added to the donor registry than in FY 2020. Due to Coronavirus Disease 2019 (COVID-19), donor recruitment organizations were not able to host in-person bone marrow donation drives at colleges and community events, which led to a decrease of approximately 9,200 donors. The inability to host in-person recruitment events adversely impacted the recruitment of all donors, including those from underrepresented and ethnically diverse populations who are less likely to join online or via digital campaigns.² These factors also contributed to a less diverse group of individuals registering in FY 2021. Despite this challenge, the CWBYCTP remained committed to identifying strategies (e.g., enhanced media outreach) to increase community engagement and donor registrations that will impact the next reporting cycle in FY 2022.

² Ahmed B. Hamed, Jessica G. Bruce, Vidya Kuniyil, Deborah L. Confer, Galen E. Switzer, Race and ethnic differences in attitudes, perceptions, and knowledge about unrelated hematopoietic stem cell donation: *A study of younger newly recruited potential donors, Transplantation and Cellular Therapy* (2022). doi: <https://doi.org/10.10.16/j.tct.2022.03.013>

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

Race/Ethnicity	FY 2020	Percentage of Total Number of Registrants Added	FY 2021	Percentage of Total Number of Registrants Added
American Indian or Alaska Native	693	0.26%	474	0.18%
Asian	12,242	4.60%	13,315	5.19%
Black or African American	10,927	4.11%	7,838	3.05%
Multi-racial	30,002	11.28%	22,557	8.79%
Native Hawaiian or Other Pacific Islander	215	0.08%	176	0.07%
Unknown ⁺	729	0.27%	5,182	2.02%
White	153,359	57.68%	156,049	60.80%
Hispanic or Latino [^]	57,695	21.70%	51,080	19.90%
Total	265,862		256,671	

Notes:

*Data Source: National Marrow Donor Program data as of February 23, 2022.

+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 may be any race.

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 four percentage points from FY 2020 to FY 2021 as shown in Table 3. The percentage of transplants facilitated across racial categories remained relatively flat. The transplant community widely believes that the unanticipated public health emergency resulted in a decrease in the number of blood stem cell transplants performed in FY 2021, a continuing trend seen in FY 2020. The “COVID-19 Impact on the CWBYCTP” section further details challenges and HRSA-supported responses by contractors.

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

Race/Ethnicity	FY 2020	Percent of Total Number of Transplants Facilitated	FY 2021	Percent of Total Number of Transplants Facilitated
American Indian or Alaska Native	35	0.57%	25	0.42%
Asian	218	3.53%	240	4.04%
Black or African American	266	4.30%	288	4.85%
Multi-racial	9	0.15%	7	0.12%
Native Hawaiian or Other Pacific Islander	9	0.15%	10	0.17%
Unknown [^]	1,291	20.88%	1,195	20.11%
White	4,356	70.44%	4,178	70.30%
Hispanic or Latino**	9	0.15%	6	0.10%
Total	6,193		5,949	

Notes:

*Data Source: NMDP data as of February 23, 2022.

+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.

**Hispanic or Latino may be any race.

Transplant Survival Rates

Because the 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 2021 target was a rate of 69 percent patient survival at 1 year post-transplant for matched unrelated blood stem cell transplants. Transplant centers generally accept the 1-year survival of first allogeneic HSCT as an outcome measure of performance, which can be obtained via the reporting tool for the Center-Specific Survival Analysis³ 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 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

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

transplant centers, along with compilation and analysis of the data, creates a lag in reporting of survival outcomes; therefore, HRSA will report the FY 2021 transplant outcomes data, including the probability of 1-year survival, in the FY 2023 annual report.

For this FY 2021 report, transplant outcomes for FY 2019 were the latest full dataset available and are reported and compared to FY 2018 data (see Table 4). The 69 percent goal was surpassed in FY 2018 and FY 2019. Survival remained relatively stable between FY 2018 and FY 2019 across the broad indications for HSCT.

Table 4: 1 year Post-transplant Survival Rate (U.S. Transplant Centers) for 2018 and 2019*

Donor Type	FY 2018	FY 2019
Unrelated	74%	74%
Matched Related	80%	80%
Mismatched Related	75%	73%

Notes:

*Data Source: CIBMTR data as of February 23, 2022.

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), Center for International Blood and Marrow Transplant Research (CIBMTR), National Marrow Donor Program (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’ decision led to the completion of a study that leveraged the reporting requirements of the SCTOD and met the criteria for the CED mechanism. The number of patients with MDS receiving transplants in the United States increased after ensuring payment coverage by CMS.

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 2021 are provided in Table 5.

These studies, which are approved under CMS’ 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 Enrolled in CMS CED Studies through September 30, 2021*

Disease Indication	Study Opened	Age	Unrelated Donor	Related Donor	Total
Myelodysplastic Syndrome	December 2010	65 and older	2,968	1,379	4,347
Myelodysplastic Syndrome	December 2010	Under 65 with Medicare	684	1,071	1,755
Myelofibrosis	December 2016	55 and older	272	109	381
Multiple myeloma	July 2017	Any	11	14	25
Sickle cell disease	November 2017	15-50	2	8	10
Total			3,937	2,581	6,518

Notes:

*Data Source: CIBMTR data as of February 23, 2022.

CWBYCTP Professional Education and Outreach Highlights

The COVID-19 pandemic impacted the delivery of education programs to medical professionals. Prior to COVID-19, many educational programs delivered in-person sessions and recorded them for online access. Due to the pandemic, beginning in March 2020 and continuing through 2021, all national and regional conferences conducted their programs virtually, if at all. The SPA-CC contractor provided professional education and outreach virtually through online meetings and pre-recorded sessions, as described below.

Transplant Referral Timing Guidelines and Resources

A timely referral is critical to patients receiving an HSCT. The SPA-CC contractor 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. A key asset in education for this audience is the Transplant Consultation Guidelines. The guidelines identify appropriate referral timing for allogeneic or autologous HSCT based on a patient’s disease characteristics. The SPA-CC contractor developed these guidelines in collaboration with ASTCT based on current clinical practice, medical literature, National Comprehensive Cancer Network® Guidelines for the treatment of cancer, and evidence-based reviews. The guidelines are available in print, online, and mobile app versions.⁴

In addition to these guidelines, the NMDP helped disseminate peer-reviewed research studies such as, “Rapid Donor Identification Improves Survival in High-Risk First-Remission Patients

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

with Acute Myeloid Leukemia,”⁵ which discusses the importance of early HLA typing and early referral to help improve patient outcomes. All research disseminated is consolidated and summarized in simple formats to ensure busy physicians can understand the data and apply them to their practice. Through these efforts and more, the NMDP is working to educate the blood stem cell community to ensure every physician has access to the latest research.

Health Professional Education Activities and Engagement

The SPA-CC and OPA contractors offered a variety of programs to educate and engage health care professionals about the need for HSCT and services offered through the CWBYCTP. Many FY 2021 professional education programs focused on sickle cell disease (SCD). HSCT is an underused treatment and the only potential cure for SCD, which primarily affects people of African descent. These initiatives were designed to address factors that may disproportionately impede access to HSCT by an underserved population.

- The SPA-CC and OPA contractors offered programs with continuing education (CE) credits for medical professionals. The ONE Forum[®] is the CWBYCTP OPA and SPA-CC contractors’ annual conference and one of the primary means of providing CE credits. The meeting held in November 2020 was a virtual event attended by a total of over 350 nurses and 350 other health care professionals (including physicians and pharmacists).
- The SPA-CC and OPA contractors also offer a variety of educational programs and resources along with CE activities to help health professionals provide the best care for patients before, during, and after transplantation. For example, the CWBYCTP developed health care professional education programs focusing on the use of HSCT in treating SCD.
- In September 2021, the OPA contractor partnered with ASTCT to provide a four-part CE series on HSCT for SCD. Referring and transplant physicians were the primary audience for this series. The secondary audience was other health professionals within an interdisciplinary team. The series covered the following topics: *Referring Patients with Sickle Cell Disease to Transplant*, *Decision Making and Preparation for Transplant*, *Barriers and Access to Transplant Care*, and *Long-Term Impact of Transplant and Survivorship*. The OPA contractor and ASTCT provided these educational opportunities via webinars and recorded them for ASTCT’s Learning Center platform. There were nearly 190 live attendees with participants able to claim CE credits for up to 1 year.
- In March 2021, the HRSA contractor co-hosted a webinar with the Sickle Cell Transplant Advocacy & Research Alliance titled *Sickle Cell Disease: Exploring Treatment Options and Psychosocial Care* that received over 40 views.
- The HRSA contractor provided the *Advances in Bone and Marrow Transplantation for Sickle Cell Disease* program through a webinar offered online for CE credits.
- To reach hematologists and oncologists nationally, HRSA provided information about SCD resources available to patients and caregivers for inclusion and publication in the

⁵ 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*, 2021; 16:6, e464-e475.

October and November 2020 and February, April, and July 2021 issues of the American Society of Hematology's SCD Coalition newsletter.

CWBYCTP Public Educational Resources and Services

Navigation Services and Education 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 search data to providers and patients. The OPA contractor's outreach program includes calls to identify patient concerns and to help remove barriers to transplant through referral to services offered by the OPA contractor, the SPA-CC contractor, and other organizations. 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 year of the program found that patients were 26 percent more likely to progress to transplant if the contractor contacted them through the outreach program.⁶

The OPA contractor continued its focus on outreach and education resources for people with SCD. This included hiring two patient navigators trained to support the unique needs of this population, creating and disseminating fact sheets, and developing an outreach package. The patient navigators created and released fact sheets on SCD therapies in early 2021 to help those living with SCD navigate conversations with health care providers on therapeutic options. They also developed an outreach package for individuals with SCD to increase awareness of treatment options and available free resources. Over 50 individuals used these packages. Additionally, the OPA contractor both mailed resources directly to SCD patients and made them publicly available through the Sick Cell Connect website.⁷ The OPA contractor also distributed materials at close to 20 events through partnerships with community groups, SCD-focused organizations, and medical centers. The OPA contractor provided the following educational resources to increase awareness and education in the Black/African American community about resources and potential cures available to treat SCD:

- The contractor distributed over 250 educational handouts at donor recruitment events and SCD advocacy events. The handouts explained what SCD is and that a bone marrow transplant is a curative option.
- The educational videos titled *My Options* and *Spencer's Sickle Cell Journey* had more than 130 views and 580 views, respectively.
- Over 200 conference attendees visited NMDP's online booth at the 8th Annual SCD Warriors Convention.

⁶ Preliminary search date October 1, 2019, to September 30, 2020; received donor/cord blood transplant facilitated by OPA contractor within 210 days of preliminary search (April 28, 2021).

⁷ www.sicklecellconnect.com (not supported in Internet Explorer).

Post-transplant survival is a primary concern of patients. The CWBYCTP contractors developed a new query tool to provide transplant survival rates specific to their situation. The tool allows users to search for survival data by disease, risk factor, transplant type, graft type, age group, sex, and patient race. This tool launched on the program website in March 2021.⁸

Summary of Published and Unpublished Studies for SCTOD

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 HSCT;
- Evaluating optimal patient selection and treatment strategies for cord blood HSCT;
- Evaluating access to care, and;
- Evaluating quality of life and late effects of allogeneic HSCT.

The SCTOD contractor's portfolio includes more than 140 unpublished studies in progress. The completed studies resulted in 65 peer-reviewed publications⁹ involving blood stem cell transplantation. The SCTOD contractor published 16 plain language or non-scientific summaries,¹⁰ surpassing the SCTOD contractor's annual goal of eight summaries designed specifically for patient use.

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

\$7 Million Funds Transfer and Identified Demonstration Projects for FY 2022

In FY 2021, \$7 million was transferred from the NCBI program to the CWBYCTP for use from October 1, 2021, through September 30, 2022. The CWBYCTP will use these funds to identify priority areas for potential future demonstration projects or special studies. In FY 2022, HRSA will provide technical assistance that will focus on cord blood selection, candidate evaluation, and sharing transplant best practices with the ultimate goal of increasing cord blood transplants. HRSA will also work to enhance the workforce in the area of cord blood by collaborating with ASTCT in sharing best practices and clinical guidelines to improve cord blood utilization and patient outcomes; support CBBs by expanding CBU collection in birthing centers; and support difficult searches by working with the ASTCT's consultants with expertise in searching for all

⁸ Survival Data Query Tool: <https://bloodstemcell.hrsa.gov/data/transplant-survival-report>.

⁹ The CIBMTR Publication List is available at <https://www.cibmtr.org/ReferenceCenter/PubList/Pages/index.aspx>.

¹⁰ The Study Summaries for Patients is available at <https://www.cibmtr.org/ReferenceCenter/Patient/PatientSummaries/pages/index.aspx>.

blood stem cell sources used in transplants, including cord blood and unrelated and related marrow donors. The consultants will teach transplant teams best practices with donor search and management. HRSA will provide any major findings resulting from these special projects in a future report to Congress.

Highlights from FY 2021 Demonstration Projects

The Stem Cell Therapeutic and Research Act of 2005, P.L. 109-129, as amended includes a requirement which 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 five FY 2021 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 the time to transplant and identify 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 advice and optimal CBU selection guidance if they do not have a fully matched adult donor option and cannot identify a suitably matched CBU. In FY 2021, there were two requests for project services, resulting in one patient receiving a transplant with the recommended CBUs.

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

The CWBYCTP provided support to NCBI contractors Bloodworks, Cleveland Cord Blood Center, Duke University, LifeSouth, and University of Texas MD Anderson Cord Blood Bank 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 necessary 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 730 CBUs (or 81 percent) out of the collection goal of more than 900 CBUs. Of this total, 64 percent were from racially diverse donors.

Support to CBBs to Perform High-Resolution Tissue Typing of Nearly 3,000 Genetically Diverse CBUs

HRSA supported the coordination of high-resolution typing on segments from nearly 3,000 CBUs at four CBBs (both NCBI and non-NCBI funded) to improve search quality and enhance matching. 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 enhance the use of the inventory. The four CBBs performed high-resolution typing using next generation sequencing on more than 2,600 CBUs. Of this total, 1,275 (or 49 percent) were from ethnically diverse donors.

Support the Recruitment and Retention of an Additional 44,530 Volunteer Adult Donors

The CWBYCTP supported the recruitment and retention of an additional 44,530 volunteer adult donors to the CWBYCTP donor registry. The program's recruitment efforts included a donor drive to increase awareness and registrations by leveraging new stories that featured ethnically diverse patients. The primary driver of these stories was paid media executed via social media channels. The program's recruitment efforts also included the launch of outreach efforts at Historically Black Colleges and Universities by working with 14 interns from those institutions.

Conduct Additional HLA Typing Tests from Underrepresented Racial and Ethnic Populations

The CWBYCTP supported efforts to tissue type nearly 8,000 volunteer adult donors from underrepresented populations. The CWBYCTP implemented these efforts through contracting with high volume HLA laboratories that had the capacity to test more than 8,000 samples per week.

COVID-19 Impact on the CWBYCTP

Throughout FY 2021, HRSA continued to provide support to the SPA-CC and OPA contractors for unanticipated expenses that resulted from the COVID-19 pandemic, including donor recruitment and retention, 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, including two pre-HSCT and post-HSCT COVID-19 fact sheets available in English and Spanish. The CWBYCTP supported the delivery of COVID-19 fact sheets to patients with a planned facilitated transplant. In addition, NMDP/Be The Match Patient Support Center held a webinar titled *Coping and Coronavirus* to provide information and answer patient questions about COVID-19.

The COVID-19 pandemic remains the primary challenge to reaching and engaging young, ethnically diverse donors for both recruitment and retention. The SPA-CC contractor continues to address this by employing social media campaigns (i.e., Facebook and Instagram) and new content such as a public service announcement TV ad to keep donors informed and inspired. Also, the SPA-CC contractor is developing customized language for donors impacted by the COVID-19 pandemic and dedicated content¹¹ to address concerns.

Support the Processing and Cryopreservation of Hematopoietic Stem Cell Products

Given the challenge of international travel due to COVID-19, HRSA provided support for the processing and cryopreservation of hematopoietic stem cell products to ensure availability for transplants. Throughout FY 2021, transplant centers made the decision to proceed with cryopreservation based on the current COVID-19 infection rate in their state, their specific hospital policies, and the patient's condition.

Cytomegalovirus Testing

HRSA supported cytomegalovirus (CMV) testing to identify the presence or absence of CMV antibodies in 50,000 potential volunteer adult donors. CMV is a common virus that can cause challenges for those with a weakened immune system; yet many people may not know they have CMV. Identifying the CMV marker can help the transplant center identify donors on the registry representing each HLA type and is most useful in scenarios where multiple potential donors exist with similar HLA matching and donor age. Identification of this marker can also help transplant centers optimize patient transplants.

Support Donor and Courier Travel

HRSA provided travel support for donors of bone marrow transplants and couriers of cord blood products after COVID-19 impacted travel capabilities. Quarantine restrictions imposed by local jurisdictions limited donors' ability to travel. Travel was also 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 keep collections' appointments as much as possible. The SPA-CC contractor also engaged several partners to continue offering private pilot aircraft and private corporate flights when commercial flights were unavailable or when the donor did not feel comfortable flying on a commercial airline.

¹¹ The SPA-CC has developed numerous webpages with COVID-19 content. One example can be found at the following URL: <https://bethematch.org/blog/patient-stories/covid-19-faqs/>

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

HRSA received emergency approval from the Office of Management and Budget to revise the data collection forms to include COVID-19 related data fields. The related additional data collected will allow the CWBYCTP to evaluate changes in HSCT practices in response to the pandemic, potential impacts on access to HSCT, and resulting outcomes at HSCT centers in the United States. The Center-Specific Survival Analysis completed in 2021¹² used this important information in its risk adjustment and HRSA anticipates it to be essential in the 2022 and 2023 analyses as well. Additionally, published studies^{13,14} have incorporated this information and are available to support policy decisions. This information may also guide future vaccination strategies or COVID treatments.

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 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 (see 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 funding decisions. When funding is available, HRSA exercises contract options to support

¹² Wingard JR, Ahn KW, Dandoy C, Perales M-A, Wood WA, Logan B, Riches M, Rizzo JD. COVID-19 and hematopoietic cell transplantation center-specific survival analysis: Can we adjust for the impact of the pandemic? Recommendations of the COVID-19 Task Force of the 2020 Center for International Blood and Marrow Transplantation Research Center Outcomes Forum. *Transplantation and Cellular Therapy*. 2021 Jul 1; 27(7): 533-539. doi: 10.1016/j.jtct.2021.04.008. Epub 2021 Apr 22. PMC8061634.

¹³ Hsu JW, Farhadfar N, Murthy H, Logan BR, Bo-Subait S, Frey N, Goldstein SC, Horowitz MM, Lazarus H, Schwanke JD, Shah NN, Spellman SR, Switzer GE, Devine SM, Shaw BE, Wingard JR. The effect of donor graft cryopreservation on allogeneic hematopoietic cell transplantation outcomes: A Center for International Blood and Marrow Transplant Research Analysis. Implications during the COVID-19 pandemic. *Transplantation and Cellular Therapy*. 2021 Jun 1; 27(6): 507-516. doi: 10.1016/j.jtct.2021.03.015. Epub 2021 Mar 22. PMC8217124.

¹⁴ Sharma A, Bhatt NS, St. Martin A, Abid MB, Bloomquist J, Chemaly RF, Dandoy C, Gauthier J, Gowda L, Perales M-A, Seropian S, Shaw BE, Tuschl EE, Zeidan AM, Riches ML, Shah GL. Clinical characteristics and outcomes of COVID-19 in haematopoietic stem-cell transplantation recipients: an observational cohort study. *The Lancet Haematology*. doi: 10.1016/S2352-3026(20)30429-4. Epub 2021 Jan 19. PMC7816949.

banking of additional CBUs. 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 2017 to 2021*

Fiscal Year	Appropriation⁺	Total Contract Award
2017	\$12,239,000	\$11,329,136
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
Total	\$79,202,000	\$65,372,756

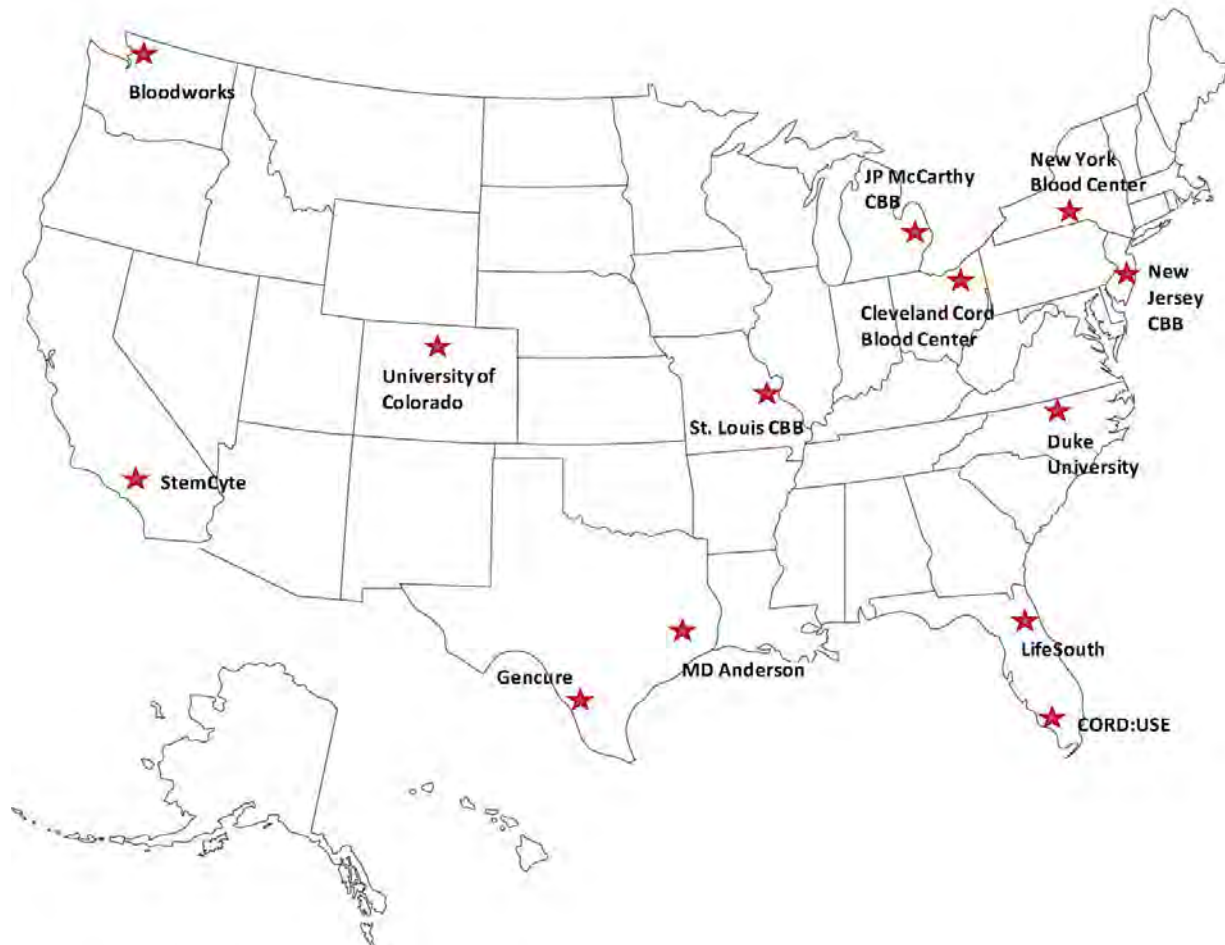
Notes:

*Data Source: Internal HRSA financial information as of February 23, 2022.

⁺Administrative costs account for differences between appropriations and total contract awards.

From the inception of the NCBI program in FY 2004 through FY 2021, HRSA awarded 28 NCBI program contracts to 13 different contractors. Figure 2 identifies organizations with contracts as of the end of FY 2021 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 2021, 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

Over 27,000 CBUs were collected from FY 2017 through FY 2021 (see Table 7), and 51 percent of CBUs shipped through the CWBYCTP were selected from NCBI (see 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. Increasing the genetic diversity of NCBI increases the chance of transplantation for those individuals who lack a suitably matched relative. Table 7 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 2020 and 2021 reflect the ongoing impacts of the COVID-19 pandemic.

As shown in Table 8, the number of non-NCBI CBUs released for transplant has decreased since FY 2017, 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 recent 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 (see Tables 3 and 8). HRSA will continue to monitor and assess trends in cord blood transplantation and share insights in future reports.

HRSA currently provides 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 2021, the amount awarded was \$16,221,529 for 4,117 CBUs, and 61 percent of these CBUs were from donors from underrepresented racial and ethnic populations.

Table 7: Contracted NCBI CBUs by Race/Ethnicity for 2017 to 2021*

Fiscal Year	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
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
2021	185	—	482	1,072	—	—	1,621	757	4,117
Total	1,371	2	4,520	7,943	2,181	0	9,223	2,185	27,425
% of Total	5%	0%	16%	29%	8%	0%	34%	8%	100%

Notes:

*Data Source: Internal HRSA information as of February 23, 2022. Data in this report may change due to delayed data responses, and modification to contracts may result in the number of CBUs funded by NCBI (also known as NCBI CBUs) to vary from prior year reports. The next report will include updated information.

⁺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.

Table 8: CBUs Released for Transplantation from 2017 to 2021*

Fiscal Year	NCBI-funded CBU Shipments	Total CBU Shipments⁺
2017	494	1,050
2018	493	949
2019	459	848
2020	344	702
2021	313	589
Total	2,103	4,138

Notes:

*Data Source: NMDP data as of February 23, 2022. **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 contractual requirements. In response to these limitations, HRSA issued the NCBI-contracted banks a “partial stop work order” which requires the contractor to stop any part of the contracted work for a mutually agreed upon time. The “partial stop work order” was specifically in regards to cord blood collection activities. HRSA instructed all CBBs that were actively collecting CBUs to halt collections in mid-April 2020. HRSA rescinded the stop work order in September 2020 for all banks except one that was unable to resume collections. This pause in contractual activities allowed the banks to reassess their circumstances in light of the adverse impacts of COVID-19. Even after collections resumed in FY 2021, the majority of CBBs experienced difficulty collecting CBUs due to the effects COVID-19. These included closure of health care facilities, collection staff shortages, and facility policies restricting non-essential staff from entering the facility for collection.

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 2021, HRSA was onboarding new members to the ACBSCT and did not hold any meetings.

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. 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).

Summary

Although the effects of COVID-19 adversely impacted the diversity of registrants in FY 2021, by increasing the overall size of the CWBYCTP donor registry, the CWBYCTP enabled thousands of transplant candidates who lack suitably matched relatives to explore viable options in identifying a matched unrelated donor. By adding thousands of CBUs to its inventory, the NCBI program also continued to play a vital role in providing transplant candidates with an additional blood stem source. Combined, both programs serve to expand access to blood stem cell transplants for those from underrepresented racial and ethnic populations and strive to achieve health equity in its operations. Over the course of 15 years, the CWBYCTP listed over 23 million volunteer adult bone marrow registrants and over 108,000 NCBI CBUs on the CWBYCTP 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.