National Marrow Donor Program®

IND ANNUAL REPORT
BB-IND #7555-0136

A Centralized Cord Blood Registry to Facilitate Allogeneic, Unrelated Donor Umbilical Cord Blood Transplantation

May 2022

I. Study Information

A. General Study Information

This report provides an annual update on the status of *BB-IND# 7555 A Centralized Cord Blood Registry to Facilitate Allogeneic, Unrelated Donor Umbilical Cord Blood Transplantation*; and the protocol under that IND: *Protocol 10-CBA: A Multicenter Access and Distribution Protocol for Unlicensed Cryopreserved Cord Blood Units (CBUs) for Transplantation in Pediatric and Adult Patients with Hematologic Malignancies and Other*

Indications. For the purposes of this report, the protocol will be referred to as Protocol 10-CBA.

Protocol 10-CBA Study Objectives

Primary Objective:

To examine the incidence of neutrophil recovery of ≥ 500/mm^3 after cord blood transplantation in a multi-institution setting using CBUs not licensed by the Food and Drug Administration (FDA).

Secondary Objectives:

- Assess the incidence of transmission of infection
- Assess the incidence of serious infusion reaction
- Determine the overall 1-year survival after cord blood transplantation
- Assess the cumulative incidence of acute GVHD grades II to IV and grades III to IV
- Assess the cumulative incidence of chronic GVHD
- Determine platelet engraftment of >20,000 mcL and >50,000 mcL

Protocol 10-CBA Patient Population

Pediatric and adult patients of any age with disorders affecting the hematopoietic system that are inherited, acquired, or result from myeloablative treatment are eligible for participation in this study.

Study Status

This annual report summarizes activity from July 1, 2020 through June 30, 2021, unless otherwise noted.

Transplant Centers (TCs) that have completed the activation process are listed in Appendix 1. Domestic and international Cord Blood Banks (CBBs) qualified as suppliers of CBUs are listed in Appendix 2. These lists are current as of April 2022 in an effort to provide current information on the status of the IND at the time of submission of this annual report.

B. Patient Enrollment

The transplant centers began enrolling patients in Protocol 10-CBA in October 2011. This study is intended to be open-ended with no maximum accrual.

As of June 30, 2021, there were 4,865 total enrollments under Protocol 10-CBA; 233 of these were enrolled between July 1, 2020 and June 30, 2021 (the reporting period). Out of the 4,865 total enrollments, 480 exited the study; 25 of these exited during the reporting period. There were 4,256 total transplants performed under the protocol on 4,166 recipients as of June 30, 2021; 218 of these 4,256 transplants were carried out during this reporting period on 217 recipients. Some patients who exited or received transplants during this reporting period may have enrolled prior to this report period. Patient enrollment and transplant activity numbers are summarized in Table 1.

The characteristics of the 217 recipients who underwent transplantation under Protocol 10-CBA during this report period are described in Appendix 3. All enrollments and transplants are included in the enrollment and activity numbers presented in Table 1. However, the patients who enrolled or received a transplant more than once are counted

only once in the demographic tables in Appendix 3 using only their first transplant information.

Table 1: Patient Enrollment and Transplant Activity

Activity	As of 6/30/20[b]	Between 7/1/20 and 6/30/21	As of 6/30/21
Signed informed consent and completed enrollment form	4,631	233	4,864
Exited the study	454	25	479
Reason participation ended			
Transplant canceled	192	8	200
Substituted non-NMDP IND cord blood unit	144	12	156
Substituted other cell source	67	3	70
Subject withdrawal	9	0	9
Ineligible	27	1	28
Other	15	1	16
Transplants under Protocol 10-CBA	4,038	218	4,256
Patients who received transplants [a]	3,949	217	4,166

[a] Patient count reflects unique individuals. Patients could receive multiple transplants, possibly over different time periods, but are counted only once.

[b] Numbers in this column may be slightly higher than listed in the 2021 annual report due to additional activity entries after data was pulled for 2021 annual report.

C. Brief Description of Study Results

Recipient outcome analysis was conducted on Protocol 10-CBA cord blood transplant recipients who underwent primary transplantation between October 20, 2011 and December 31, 2020 and for whom the NMDP had received complete survival outcome data at the time of the data pull for this report. The cut-off date for inclusion in the analysis was December 31, 2020 to allow for sufficient follow-up on recipient outcomes. The final analysis consisted of 3,604 primary cord blood transplant recipients. The number of 10-CBA transplant recipients after applying each exclusion criterion are listed in Table 2.

Table 2: Inclusion/Exclusion Criteria

Selection Criteria	N
10-CBA transplants between 10/20/2011 and 12/31/2020	4,163
Primary transplants	3,612
Complete survival outcome data received	3,604

The analysis was performed separately for adult patients with malignant disease, adult patients with non-malignant disease, pediatric patients with malignant disease, and pediatric patients with non-malignant disease. Pediatric patients are defined as individuals younger than 13 years of age and adult is defined as older than or equal to 13 years of age. The age cut-off is used because patient weight distributions ultimately determine cord blood cell dose which is a factor known to affect outcomes. Malignant diseases include leukemias, myelodysplastic/myeloproliferative disorders, lymphomas, plasma cell disorders, and solid tumors. Non-malignant diseases include immune deficiencies, metabolic disorders, other inherited abnormalities, severe aplastic anemia, histiocytic disorders, and autoimmune diseases.

Two primary outcomes, overall survival and neutrophil engraftment were examined. Univariate probabilities of overall survival were calculated using the Kaplan-Meier estimator. Neutrophil engraftment is defined as achievement of an absolute neutrophil count (ANC) of $\geq$ 500 neutrophils/mm^3 sustained for three consecutive laboratory measurements on different days. Patients who received a reduced intensity transplant or whose ANC never went below 500 neutrophils/mm^3 were excluded from the neutrophil engraftment analysis. A conditioning regimen was considered myeloablative if it included one of the following:

- Total Body Irradiation (TBI) > 500 cGy as a single fraction
- TBI > 800 cGy regardless of the number of fractions
- Busulfan + Cyclophosphamide
- Busulfan + Melphalan
- Busulfan + Thiotepa
- Melphalan + Thiotepa
- Cyclophosphamide + Thiotepa
- Busulfan PO ≥ 9 mg/kg or IV ≥ 7.2 mg/kg
- Melphalan > 150 mg/m^2
- Treosulfan > 30000 mg/ m^2
- Thiotepa ≥ 10 mg/kg

All other conditioning regimens were considered reduced intensity.

Probability of neutrophil recovery was calculated using the cumulative incidence function estimator with death without an event as the competing risk and a subsequent transplant as a censoring event. All confidence intervals were calculated with the use of an arcsine-square root transformation.

Transplant Recipient Characteristics

The characteristics of the 3,604 primary cord blood transplant recipients with complete survival data are presented in Table 4-1 of Appendix 4. In total, males comprised 55% of all recipients. Caucasians comprised 50% of the total recipients in this analysis. The median age at transplant was 30 years for all recipients, with the youngest recipient being under the age of one year and the oldest recipient being 82 years old. Patients with various forms of leukemia comprised 66% of all recipients. Patients with diseases

including myelodysplastic disorders, lymphoma, and other malignancies comprised 16% of total recipients. The remaining 18% of all patients were treated for immune deficiencies, metabolic disorders, severe aplastic anemia, or other non-malignant indications.

Adult recipients with malignant diseases included 53% males while adult recipients with non-malignant diseases included 49% males. Caucasians represented 55% and 44% of adult recipients with malignant diseases and non-malignant diseases. The median age of adults with malignant diseases was 47 years, ranging between 13 and 82 years of age. The median age of adult recipients with non-malignant disease was lower than adults with malignant disease at 21 years, ranging between 13 and 66 years of age. Patients with various forms of leukemia comprised 77% of adult recipients, higher than the percentage of the total population. Patients with diseases including myelodysplastic disorders, lymphoma, and other malignancies comprised 20% of adult recipients. The remaining 3% of adult patients were treated for immune deficiencies, metabolic disorders, severe aplastic anemia, or other non-malignant indications.

Males made up 56% of the pediatric recipients with malignant diseases and 62% of pediatric recipients with non-malignant diseases. Pediatric recipients displayed a lower percentage of Caucasians than in the total population (39% in recipients with malignant disease and 45% in recipients with non-malignant disease). The median age at transplant was 5 years for children with malignant diseases and 1 year for children with non-malignant diseases; both groups displayed ranges from less than one year for the youngest to 12 years of age for the oldest. Overall, fewer pediatric patients suffered from leukemia (47%) and other malignant diseases (6%),but presented with many more non-malignant conditions (47%) compared to adult recipients.

Transplant Recipient Outcomes

Univariate probabilities of transplant outcomes after primary cord blood transplantation by age-disease category are presented in Table 4-2 in Appendix 4. Figure 4-1 and Figure 4-2 of Appendix 4 display univariate curves of these outcomes. However, caution should be employed when evaluating these outcomes due to the limited sample sizes, particularly in the adult non-malignant group, and because the analyses were not adjusted for many other factors that could affect the outcomes.

Overall Survival

Overall Survival at one year was 62% (95% CI = 60-64) for adults with malignant diseases, 59% (95% CI = 47-71) for adults with non-malignant diseases, 72% (95% CI = 68-75) for children with malignant diseases, and 83% (95% CI = 80-86) for children with non-malignant diseases.

Neutrophil Engraftment

Neutrophil engraftment rate at 100 days was 93% (95% CI = 92-95) for adults with malignant diseases, 87% (95% CI = 68-98) for adults with non-malignant diseases, 93% (95% CI = 91-95) for children with malignant diseases, and 95% (95% CI = 93-97) for children with non-malignant diseases.

Incidence of GVHD

The summary of our experience with umbilical cord blood transplant was published in April 2020.* The incidence of acute GVHD Grades II-IV was 35% (95% confidence interval [CI]: 33–38%), 32% (95% CI: 28–36%), and 24% (95% CI:19–28%) for adults, pediatric malignant and pediatric non-malignant cohorts, respectively. Acute GVHD grades III-IV

was low at 16% (95%CI: 15–20%), 17% (95%CI: 14–18%), and 9% (95%CI: 6–12%) for adults, pediatric malignant and pediatric non-malignant, respectively. Chronic GVHD (limited and extensive) at one year was 24% (95% CI: 22–27%), 26% (95% CI: 22–30%), and 24% (95% CI: 20–28%) for adults, pediatric malignant, and pediatric non-malignant respectively. Of those that developed Chronic GVHD, 61% were classified as extensive.

*Ballen K, Logan B, Chitphakdithai P, et al. *Unlicensed umbilical cord blood units provide a safe and effective graft source for a diverse population: a study of 2456 umbilical cord blood recipients*. Biol Blood Marrow Transplant. 2020 April ; 26(4): 745–757. doi:10.1016/j.bbmt.2019.11.016

Summary Information

A. Assessed as Serious Adverse Events

There was one serious event reported to NMDP between July 1, 2020 and June 30, 2021. This event was reported by an Investigator at a participating TC and was also assessed as serious by the NMDP Medical Monitor. This was considered an expected event and is summarized in Table 3.

Table 3: Assessed Serious Adverse Events

Description of Adverse Event	Number of Events
Grade 4 Hypertension in a pediatric recipient. Managed with medication. Recovered.	**1**
Total Number of Events	**1**

B. Adverse Events (AE)/Symptoms Associated with Infusions

Out of 218 transplants between July 1, 2020 and June 30, 2021, 193 had completed the adverse events (AE) question on the *CIBMTR Hematopoietic Cellular Transplant (HCT) Infusion Form*. Adverse events associated with infusion were reported by the TC as part of the standard CIBMTR outcome reporting on the HCT Infusion Form. Of the 193 reports, there were 38 (20%) transplants with adverse experiences manifesting in various symptoms; 24 reported 1 symptom, 7 reported 2 symptoms, 4 reported 3 symptoms, 1 reported 4 symptoms, and 2 reported 5 or more symptoms. The details of these adverse events, as reported by the TCs are summarized in Table 4.

Table 4: Reported Adverse Events / Symptoms

	Adverse Event Occurrence		Symptom Result of Infusion?	
Symptoms	**Present**	**Absent**	**Yes**	**No**
Bradycardia	1	37	1	0
Chest tightness/pain	3	35	3	0
Chills at time of infusion	0	38	N/A	N/A
Fever <= 103F within 24 hrs of infusion	1	37	0	1
Gross hemoglobinuria	1	37	1	0
Headache	2	36	2	0
Hives	0	38	N/A	N/A
Hypertension	33	5	32	1

Hypotension	0	38	N/A	N/A
Hypoxia requiring oxygen support	2	36	2	0
Nausea	9	29	6	3
Rigors, mild	0	38	N/A	N/A
Rigors, severe	0	38	N/A	N/A
Shortness of breath (SOB)	1	37	1	0
Tachycardia	1	37	0	1
Vomiting	5	33	4	1
Other expected AE[a]	4	34	4	0
Other unexpected AE[b]	1	37	1	0

[a] Numbness, bronchospasm, diarrhea, back pain

[b] Acute perfusion changes

C. IND Safety Reports

There were no IND safety reports submitted between 7/1/2020 and 6/30/2021.

D. Patient Deaths

Out of 4,166 patients who underwent transplantation under Protocol 10-CBA as of June 30, 2021, 4,091 had known survival status at the time of the data pull for this report. Among these 4,091 patients, 1,880 patient deaths were reported.

E. Dropouts Associated with Adverse Events

No enrolled patients have been reported to drop out because of an adverse event, during the reporting period.

F. Brief Description of Learned Information

There are no study results to report during the reporting period.

G. Deviations and Complaints Related to Manufacturing of Distributed Product

Deviations and product complaints are investigated, documented and reviewed in the Quality Management System. While CBUs distributed under 10-CBA are not licensed products, the table below include the events reported from July 1, 2020 to June 30, 2021 that would meet BPD reporting criteria for licensed biological products.

Reference: Biological Product Deviation Reporting and HCT/P Deviation Reporting. Non blood BPD Reporting Codes. https://www.fda.gov/media/142562/download

Table 5: Deviations and Complaints Related to Manufacturing for Distributed Products

Description	Number of Events	BPD Code
TC (Consignee) reported a CBU bag leak during thaw. Product salvaged, sampled for sterility and	1	QC-64-04

Description	Number of Events	BPD Code
infused without adverse event. Sterility result was negative. TC indicated the CBU was received from CBB in acceptable condition, and no issues were identified with the dry shipper upon receipt nor packing of the CBU inside the shipper. TC inspected and stored product in LN2 freezer upon receipt. CBB responsible for notifying cryobag manufacturer and confirmed this was done. Indeterminable whether bag failure was due to handling or manufacturer defect.		
Incident #1 TC (Consignee) reported upon receipt of the international CBU that and the product was thawed and not useable. Back up CBU requested and infused. Investigation concluded dry shipper was likely damaged during shipment, experienced high external temperatures. CBB removed dry shipping unit from service pending revalidation. SOP changes implemented at CBB. Incident #2 TC (Consignee) reported that the CBU unit arrived thawed and not useable. No liquid nitrogen vapor remaining in the dry shipper. Back up unit requested and infused. Investigation concluded the dry shipper may have been transported upside down or on its side, resulting loss of LN2 and decreased hold time. Logistics team notified for evaluation and consideration in commercial courier -selection.	2	QC-62-02

TC = Transplant center
CBU = Cord Blood Unit
CBB = Cord Blood Bank
LN2= Liquid Nitrogen

H. Preclinical Studies and Major Preclinical Studies

There are no preclinical studies underway during this reporting period.

I. Significant Manufacturing and/or Microbiological Changes

There were no significant manufacturing or microbiological changes during this reporting period.

II. General Investigational Plan for the Coming Year

It is expected that enrollment will continue at a rate that is consistent with previous years' pace. Study endpoints and additional interim data analysis will be provided, if statistically significant numbers are reported to NMDP and/or CIBMTR.

III. Summary of Investigator Brochure Revision(s)

There were no revisions to the Investigator Brochure during the reporting period. The current version has been included with this submission for reference.

IV. Significant Phase I Protocol Modifications

There were no protocol amendments during the reporting period. Version 10.3 was approved by the NMDP IRB on September 19, 2019.

V. Summary of Significant Foreign Market Developments

None

VI. Other Information

The NMDP will continue to submit annual cost recovery information as a separate Amendment to IND BB-7555.

VII. Outstanding Business

No outstanding business at the time of this report.

Appendix 1

Protocol 10-CBA Activated Transplant Centers

Table 1-1: Transplant Centers Activated under Protocol 10-CBA (current as of 4/04/2022)

Transplant Center	City	State
University of Alabama at Birmingham	Birmingham	AL
University of Arizona Medical Center – Tucson	Tucson	AZ
Mayo Clinic Arizona (Adult)	Phoenix	AZ
Phoenix Children's Hospital	Phoenix	AZ
Banner MD Anderson Cancer Center	Gilbert	AZ
Rady Children's Hospital San Diego	San Diego	CA
Loma Linda University Cancer Center	Loma Linda	CA
University of California San Diego	La Jolla	CA
Children's Hospital of Oakland	Oakland	CA
Scripps Blood and Marrow Transplant Program	La Jolla	CA
Children's Hospital of Los Angeles	Los Angeles	CA
University of California San Francisco (Adult and Pediatric)	San Francisco	CA
City of Hope National Medical Center	Duarte	CA
Children's Hospital of Orange County	Orange	CA
Sutter Medical Center	Sacramento	CA
UCSF Med Center Adults	San Francisco	CA
University of California Los Angeles (UCLA)	Los Angeles	CA
Lucile Packard Children's Hospital	Stanford	CA
Stanford University Medical Center (Adults)	Stanford	CA
Children's Hospital Colorado	Aurora	CO
Presbyterian/St. Luke's/Colorado Blood Cancer Institute	Denver	CO
University of Colorado Hospital	Aurora	CO
Yale University School of Medicine/Yale New Haven Hospital	New Haven	CT
Children's National Medical Center	Washington	DC
Nemours Children's Hospital - Alfred I. duPont Hospital for Children	Wilmington	DE
Mayo Clinic Jacksonville (Adult)	Jacksonville	FL
Nemours Children's Hospital (Mayo Jacksonville - Pediatric)	Jacksonville	FL
Nicklaus Children's Hospital	Miami	FL
University of Miami - ADULTS	Miami	FL
University of Florida Shands Cancer Center	Gainesville	FL
All Children's Hospital	St. Petersburg	FL
H. Lee Moffitt Cancer Center	Tampa	FL
AdventHeatlh Orlando BMT Center - Adults	Orlando	FL
University of Miami (Pediatric)	Miami	FL
Florida Center for Pediatric Cellular Therapy	Orlando	FL
Children's Healthcare of Atlanta	Atlanta	GA
Northside Hospital	Atlanta	GA
Medical College of Georgia	Augusta	GA
Kapi'olani Medical Center for Women and Children	Honolulu	HI
University of Iowa Hospitals and Clinics	Iowa City	IA

Transplant Center	City	State
Ann and Robert H. Lurie Children's Hospital of Chicago	Chicago	IL
Loyola University Medical Center	Maywood	IL
University of Illinois at Chicago	Chicago	IL
Northwestern Memorial Hospital	Chicago	IL
Rush University Medical Center	Chicago	IL
Advocate Lutheran General Hospital	Park Ridge	IL
Cancer Treatment Centers of America	Zion	IL
Comer Children's Hospital (Pediatric)	Chicago	IL
University of Chicago Medicine (Adults)	Chicago	IL
Indiana Blood and Marrow Transplantation, LLC (St. Francis)	Indianapolis	IN
Indiana University/Riley Hospital for Children	Indianapolis	IN
University of Kansas	Westwood	KS
University of Louisville Hospital - James Brown Cancer Center	Louisville	KY
Louisiana State University Health Sciences Ctr/ Children's Hospital of New Orleans	New Orleans	LA
Tulane Medical Center	New Orleans	LA
University of Massachusetts	Worcester	MA
Beth Israel Deaconess Medical Center	Boston	MA
Dana Farber Cancer Institute (Adult)	Boston	MA
Tufts Medical Center	Boston	MA
Massachusetts General Hospital	Boston	MA
Dana Farber Cancer Institute (Pediatric)	Boston	MA
National Institutes of Health	Bethesda	MD
Greenebaum Cancer Center/University of Maryland	Baltimore	MD
The Sidney Kimmel Comprehensive Cancer Center at John's Hopkins	Baltimore	MD
Henry Ford Hospital Bone Marrow Transplant Program	Detroit	MI
Helen DeVos Children's Hospital	Grand Rapids	MI
Karmanos Cancer Institute	Detroit	MI
University of Michigan Medical Center	Ann Arbor	MI
Children's Hospital of Michigan	Detroit	MI
Spectrum Health	Grand Rapids	MI
Mayo Clinic Rochester	Rochester	MN
Cardinal Glennon Children's Hospital	St. Louis	MO
Barnes Jewish Hospital	St. Louis	MO
St. Louis University Hospital	St. Louis	MO
Children's Mercy Hospital and Clinics	Kansas City	MO
Wash U/St. Louis Children's Hospital	St. Louis	MO
University of Mississippi Medical Center	Jackson	MS
University of North Carolina	Chapel Hill	NC
Duke University Medical Center (Pediatric)	Durham	NC
Duke University Medical Center (Adults)	Durham	NC
Levine Children's Hospital	Charlotte	NC
University of Nebraska Medical Center	Omaha	NE

Transplant Center	City	State
Robert Wood Johnson University Hospital/CINJ	New Brunswick	NJ
Hackensack University Medical Center	Hackensack	NJ
New York Presbyterian Hospital - Weill Cornell	New York	NY
Columbia University Medical Center - Morgan Stanley Children's Hospital	New York	NY
Cohen Children's Medical Center of New York	New Hyde Park	NY
Strong Memorial Hospital/University of Rochester	Rochester	NY
Roswell Park Cancer Institute	Buffalo	NY
North Shore University Hospital	Lake Success	NY
Mount Sinai Medical Center	New York	NY
Westchester Medical Center	Valhalla	NY
NYU Langone Medical Center	New York	NY
Stony Brook University Hospital	New York	NY
Memorial Sloan-Kettering Cancer Center (Adult)	New York	NY
Memorial Sloan-Kettering Cancer Center (Pediatric)	New York	NY
Children's Hospital at Montefiore Medical Center	Bronx	NY
Nationwide Children's Hospital	Columbus	OH
Cleveland Clinic Foundation	Cleveland	OH
Arthur G. James Cancer Hospital/The Ohio State University Medical Center	Columbus	OH
Cincinnati Children's Hospital	Cincinnati	OH
University Hospitals Case Medical Center	Cleveland	OH
Children's Hospital Medical Center of Akron	Akron	OH
University of Oklahoma Health Sciences Center	Oklahoma City	OK
Oregon Health & Science University (Adult)	Portland	OR
Doernbecher Children's Hospital OHSU (Peds)	Portland	OR
University of Pennsylvania Medical Center	Philadelphia	PA
Penn State Milton S. Hershey Medical Center	Hershey	PA
University of Pittsburgh Medical Center	Pittsburgh	PA
Children's Hospital of Philadelphia	Philadelphia	PA
Western Pennsylvania Cancer Institute	Pittsburgh	PA
Children's Hospital of Pittsburgh	Pittsburgh	PA
Roger Williams Medical Center	Providence	RI
Medical University of South Carolina	Charleston	SC
Avera McKennon Transplant Institute	Sioux Falls	SD
Vanderbilt University Medical Center	Nashville	TN
Sarah Cannon BMT Program	Nashville	TN
St. Jude Children's Research Hospital	Memphis	TN
Methodist Healthcare Blood and Marrow Transplant Center	Memphis	TN
Texas Transplant Institute	San Antonio	TX
Children's Medical Center of Dallas	Dallas	TX
Medical City Dallas Hospital	Dallas	TX
Baylor College of Medicine	Houston	TX
M.D. Anderson Cancer Center	Houston	TX
Cook Children's Hospital	Fort Worth	TX

Transplant Center	City	State
Zachary and Elizabeth M. Fisher Bone Marrow Transplant Program/Brooke Army Medical Center	Fort Sam Houston	TX
The Children's Hospital of San Antonio	San Antonio	TX
University of Utah (Adult)	Salt Lake City	UT
University of Utah (Peds)	Salt Lake City	UT
Virginia Commonwealth University	Richmond	VA
UVA Health System	Charlottesville	VA
Fred Hutchinson Cancer Research Center	Seattle	WA
VA Puget Sound Health Care System	Seattle	WA
University of Wisconsin Hospital & Clinics	Madison	WI
Medical College of Wisconsin	Milwaukee	WI
Children's Hospital of Wisconsin	Milwaukee	WI
West Virginia University Hospitals, Inc.	Morgantown	WV

Appendix 2

Protocol 10-CBA Qualified Cord Blood Banks

Table 2-1: Qualified US Cord Blood Banks (Current as of 3/27/2022)

Name of Cord Blood Bank	City	State
Celebration Stem Cell	Gilbert	AZ
StemCyte Inc.	Baldwin Park	CA
Children's Hospital of Orange County	Orange	CA
San Diego Blood Bank	San Diego	CA
University of Colorado Cord Blood Bank	Aurora	CO
St. Louis Cord Blood Bank (Inventory only-maintained at Univ of CO Cord Blood	Aurora	CO
Cord for Life	Altamonte Springs	FL
LifeSouth Cord Blood Bank	Gainesville	FL
Cryo-Cell International Cord Blood Bank	Oldsmar	FL
J.P. McCarthy Cord Stem Cell Bank	Detroit	MI
Versiti Cord Blood Bank (Michigan Blood)	Grand Rapids	MI
Carolinas Cord Blood Bank	Durham	NC
ITxM Clinical Services Cord Blood Lab (Inventory only- maintained at Vitalant)	Allendale	NJ
Vitalant (Allendale)	Allendale	NJ
Lifebank USA / Celgene	Florham Park	NJ
New York Blood Center	Long Island City	NY
Cleveland Cord Blood Center	Warrensville Heights	OH
M.D. Anderson Cord Blood Bank	Houston	TX
Texas Cord Blood Bank	San Antonio	TX
Bloodworks	Seattle	WA

Table 2-2: Qualified International Cord Blood Banks (Current as of 3/27/2022)

Name of Cord Blood Bank	City	Country
Banco Público de Referencia Nacional de Sangre de Cordón Umbilical	Buenos Aires	Argentina
Queensland Cord Blood Bank At The Mater	Brisbane	Australia
Sydney Cord Blood Bank	Randwick	Australia
BMDI Cord Blood Bank	Victoria	Australia
Bordet-ULB Cord Blood Bank	Brussels	Belgium
UCL Cord Blood Bank	Brussels	Belgium
CBB UZ-Gent/IMS Red Cross Flanders	Gent	Belgium
Liege Cord Blood Bank	Liege	Belgium
Leuvense Navelstrengbloedbank UZ Leuven	Leuven	Belgium
Instituto Nacional de Cancer	Rio de Janeiro	Brazil
Victoria Angel Registry of Hope	Markham	Canada
Canadian Blood Services National Public CBB	Ottawa	Canada
Hema-Quebec	Montreal	Canada
VidaCel	Santiago	Chile
Finnish Bone Marrow Donor Registry	Helsinki	Finland
EFS De Bourgogne Franche-Comte	Besancon	France
Hopital Saint-Louis	Paris	France
EFS Ile France Site De Creteil	Creteil	France
EFS Aquitaine-Limousin	Bordeaux	France
Hopital Saint-Eloi	Montpellier	France
Hopital Saint-Eloi	Lyon	France
Institut Paoli Calmettes	Marseille	France
EFS Poitou Charente Site De Poitiers- La Milentrie	Poitiers	France
EFS Nord de France site de Lille	Lille	France
EFS Bretagne Site De Rennes	Rennes	France
CHU de Brabois	Nancy	France
José Carreras Cord Blood Bank Düsseldorf	Dusseldorf	Germany
Bayerische Stammzellbank gGmbH	Gauting	Germany
DKMS Lifeline Cord Blood Bank	Dresden	Germany
University Hospital Erlangen Department of Transfusion Medicine and Haemostaseology	Erlangen	Germany
DRK Blutspendedienst Baden Wuerttemberg-Hessen gGmbH Nabelschnurblutbank Mannheim Inst.	Mannheim	Germany
Hellenic Cord Blood Bank- Biomedical Research Foundation, Academy of Athens	Athens	Greece
Jeevan Blood Bank and Research Centre	Chennai	India
Sheba Cord Blood Bank	Tel Hashomer	Israel
MDA Public Cord Blood Bank	Ramat Gan	Israel
Bedomaich Chayi Cord Blood Bank	Jerusalem	Israel
Milano Cord Blood Bank	Milano	Italy
Torino Cord Blood Bank	Torino	Italy
Banca del Cordone Ombelicale	Firenze (Florence)	Italy
Banca del Sangue di Cordone Ombelicale (SCO)della Regione Lazio	Roma	Italy
Padova Cord Blood Cord Bank	Padova	Italy
Pavia Cord Blood Bank	Pavia	Italy
Treviso Cord Blood Bank	Treviso	Italy
Pescara Cord Blood Bank	Pescara	Italy
Emilia-Romagna Cord Blood Bank (ERCB)	Bologna	Italy
UNICATT Cord Blood Bank	Roma	Italy
Pisa Cord Blood Bank	Pisa	Italy

Name of Cord Blood Bank	City	Country
Liguria Cord Blood Bank	Genova	Italy
BaSCO Campania CBB - AORN Santobono-Pausilipon	Napoli	Italy
Calabria Cord Blood Bank	Reggio Calabria	Italy
Cagliari Cord Blood Bank	Cagliari	Italy
Verona Cord Blood Bank	Verona	Italy
Sicily Cord Blood Bank	Sicily	Italy
Puglia Cord Blood Bank	S. Giovanni Rotondo (FG)	Italy
Sanquin Cord Blood Bank	Leiden	Netherlands
Singapore Cord Blood Bank, Ltd.	Singapore	Singapore
Slovenský register placentárnych krvotvorných buniek (S.R.P.K.B) Eurocord-Slovakia	Bratislava	Slovakia
Programa Concordia Banc de Sang i Teixits	Barcelona	Spain
FUNDACION PÚBLICA CENTRO DE TRASNFUSIÓN DE GALICIA	Santiago de Compostela	Spain
Cord Blood Bank. Transfusion Center. Madrid	Madrid	Spain
Cord Blood Bank Andalucia (Malaga)	Malaga	Spain
Centro Vasco de Transfusion y Tejidos Humanos	Galdakao	Spain
Banco de Cordon de la Comunidad Valenciana	Valencia	Spain
The Swedish National Cord Blood Bank	Gothenburg	Sweden
Geneva Cord Blood Bank	Geneva	Switzerland
Cord Blood Bank Basel	Basel	Switzerland
StemCyte Taiwan Co. Ltd.	LinKou	Taiwan
HealthBanks BioTech Co., Ltd.	Taipei	Taiwan
CBB/Hematopoietic Stem Cell Res. Lab, Koo Foundation Sun Yat-Sen Canc Ctr, Taipei, Taiwan	Taipei	Taiwan
Ankara University Cord Blood Bank	Ankara	Turkey
NHS Cord Blood Bank	Bristol	UK
Anthony Nolan Cord Blood Bank	Nottingham	UK

Appendix 3

Recipient Characteristics
7/1/2020 – 6/30/2021

Table 3-1: Characteristics of recipients who underwent cord blood transplantation under Protocol 10-CBA between 7/1/2020 and 6/30/2021

Variable	N	(%)
Number of Recipients	217	
Sex		
Female	106	(49)
Male	111	(51)
Race/Ethnicity		
Caucasian	94	(43)
Hispanic	52	(24)
Black/African American	31	(14)
Asian/Pacific Islander	15	(7)
American Indian/Alaska Native	1	(<1)
Other/Multiple Race	3	(1)
Decline/Unknown	21	(10)
Age		
0 to 4	57	(26)
5 to 12	22	(10)
13 to 19	14	(6)
20 to 29	14	(6)
30 to 39	30	(14)
40 to 49	22	(10)
50 to 59	24	(11)
60 to 64	12	(6)
65 to 69	18	(8)
70 or older	4	(2)
Median (Range)	30	(0-73)

Table 3-1: Characteristics of recipients who underwent cord blood transplantation under Protocol 10-CBA between 7/1/2020 and 6/30/2021 (Continued)

Variable	N	(%)
Disease		
Acute Myelogenous Leukemia (AML)	74	(34)
Acute Lymphoblastic Leukemia (ALL)	50	(23)
Other Acute Leukemia	5	(2)
Chronic Myelogenous Leukemia (CML)	5	(2)
Chronic Lymphocytic Leukemia (CLL) and Other Chronic Leukemia	2	(1)
Myelodysplastic Disorders (MDS)	20	(9)
Non-Hodgkin Lymphoma (NHL)	10	(5)
Hodgkin Lymphoma (HL)	0	(0)
Plasma Cell Disorders	1	(<1)
Severe Aplastic Anemia	0	(0)
Inherited Erythrocyte Abnormalities	9	(4)
Inherited Immune System Disorders	14	(6)
Inherited Metabolism Disorders	22	(10)
Histiocytic Disorders	1	(<1)
Inherited Platelet Abnormalities	1	(<1)
Other Non-Malignant Diseases	3	(1)

Appendix 4

Primary Transplant Analysis
10/20/2011 – 12/31/2020

Table 4-1: Characteristics of recipients who underwent primary cord blood transplantation under Protocol 10-CBA between 10/20/2011 and 12/31/2020 by age-disease category

Variable	Adult (13 Or older) With Malignant Disease		Adult (13 or older) With Non-Malignant Disease		Pediatric (Under 13) With Malignant Disease		Pediatric (Under 13) With Non-Malignant Disease	
	N	(%)	N	(%)	N	(%)	N	(%)
Number of Recipients	2317		59		655		575	
Follow-up among survivors (Months)								
N Survivors	1095		30		411		458	
Median (Range)	49	(1-121)	50	(11-96)	55	(4-121)	57	(3-119)
Sex								
Male	1236	(53)	29	(49)	368	(56)	359	(62)
Female	1081	(47)	30	(51)	287	(44)	216	(38)
Race/Ethnicity								
Caucasian	1272	(55)	26	(44)	257	(39)	259	(45)
Hispanic	448	(19)	14	(24)	227	(35)	155	(27)
Black/African American	296	(13)	10	(17)	88	(13)	83	(14)
Asian/Pacific Islander	208	(9)	5	(8)	26	(4)	30	(5)
American Indian/Alaska Native	12	(1)	0		7	(1)	6	(1)
Other/Multiple Race	23	(1)	1	(2)	24	(4)	20	(3)
Decline/Unknown	58	(3)	3	(5)	26	(4)	22	(4)
Age								
0 to 4	N/A	N/A	N/A	N/A	314	(48)	456	(79)
5 to 12	N/A	N/A	N/A	N/A	341	(52)	119	(21)
13 to 19	244	(11)	27	(46)	N/A	N/A	N/A	N/A
20 to 29	286	(12)	15	(25)	N/A	N/A	N/A	N/A
30 to 39	376	(16)	6	(10)	N/A	N/A	N/A	N/A
40 to 49	351	(15)	5	(8)	N/A	N/A	N/A	N/A
50 to 59	496	(21)	5	(8)	N/A	N/A	N/A	N/A
60 to 64	272	(12)	0		N/A	N/A	N/A	N/A
65 to 69	221	(10)	1	(2)	N/A	N/A	N/A	N/A
70 or more	71	(3)	0		N/A	N/A	N/A	N/A
Median (Range)	47	(13-82)	21	(13-66)	5	(0-12)	1	(0-12)

Table 4-1: Characteristics of recipients who underwent primary cord blood transplantation under Protocol 10-CBA between 10/20/2011 and 12/31/2020 by age-disease category (Continued)

Variable	Adult (13 Or older) With Malignant Disease		Adult (13 or older) With Non-Malignant Disease		Pediatric (Under 13) With Malignant Disease		Pediatric (Under 13) With Non-Malignant Disease	
	N	(%)	N	(%)	N	(%)	N	(%)
Disease								
Acute Myelogenous Leukemia (AML)	1164	(50)	N/A	N/A	265	(40)	N/A	N/A
Acute Lymphoblastic Leukemia (ALL)	516	(22)	N/A	N/A	283	(43)	N/A	N/A
Other Acute Leukemia	31	(1)	N/A	N/A	21	(3)	N/A	N/A
Chronic Myelogenous Leukemia (CML)	79	(3)	N/A	N/A	3	(<1)	N/A	N/A
Chronic Lymphocytic Leukemia (CLL) and Other Chronic Leukemia	46	(2)	N/A	N/A	2	(<1)	N/A	N/A
Myelodysplastic Disorders (MDS)	283	(12)	N/A	N/A	75	(11)	N/A	N/A
Non-Hodgkin Lymphoma (NHL)	178	(8)	N/A	N/A	6	(1)	N/A	N/A
Hodgkin Lymphoma (HL)	17	(1)	N/A	N/A	0	(0)	N/A	N/A
Plasma Cell Disorders	3	(<1)	N/A	N/A	0	(0)	N/A	N/A
Severe Aplastic Anemia	N/A	N/A	26	(44)	N/A	N/A	23	(4)
Inherited Erythrocyte Abnormalities	N/A	N/A	13	(22)	N/A	N/A	81	(14)
Inherited Immune System Disorders	N/A	N/A	5	(8)	N/A	N/A	209	(36)
Inherited Metabolism Disorders	N/A	N/A	5	(8)	N/A	N/A	217	(38)
Histiocytic Disorders	N/A	N/A	4	(7)	N/A	N/A	29	(5)
Inherited Platelet Abnormalities	N/A	N/A	2	(3)	N/A	N/A	10	(2)
Other Nonmalignant Diseases	N/A	N/A	4	(7)	N/A	N/A	6	(1)

Table 4-2: Univariate probabilities of transplant outcomes among recipients after primary cord blood transplantation under Protocol 10-CBA between 10/20/2011 and 12/31/2021 by age-disease category

Outcome Event	Adult (13 Or older) With Malignant Disease			Adult (13 or older) With Non-Malignant Disease			Pediatric (Under 13) With Malignant Disease			Pediatric (Under 13) With Non-Malignant Disease		
	N	Prob* (%)	95% C.I.*	N	Prob (%)	95% C.I.	N	Prob (%)	95% C.I.	N	Prob (%)	95% C.I.
Overall Survival	2315			59			655			575		
@ 100 days		84	(83-86)		86	(77-94)		88	(86-91)		92	(90-94)
@ 6 months		74	(72-76)		81	(71-90)		81	(78-84)		86	(83-89)
@ 1 year		62	(60-64)		59	(47-71)		72	(68-75)		83	(80-86)
Neutrophil Engraftment (Myeloablative only)	1112			23			628			402		
@ 60 days		93	(91-94)		87	(68-98)		92	(89-94)		94	(91-96)
@ 100 days		93	(92-95)		87	(68-98)		93	(91-95)		95	(93-97)

*Prob = Probability, C.I. = Confidence Interval

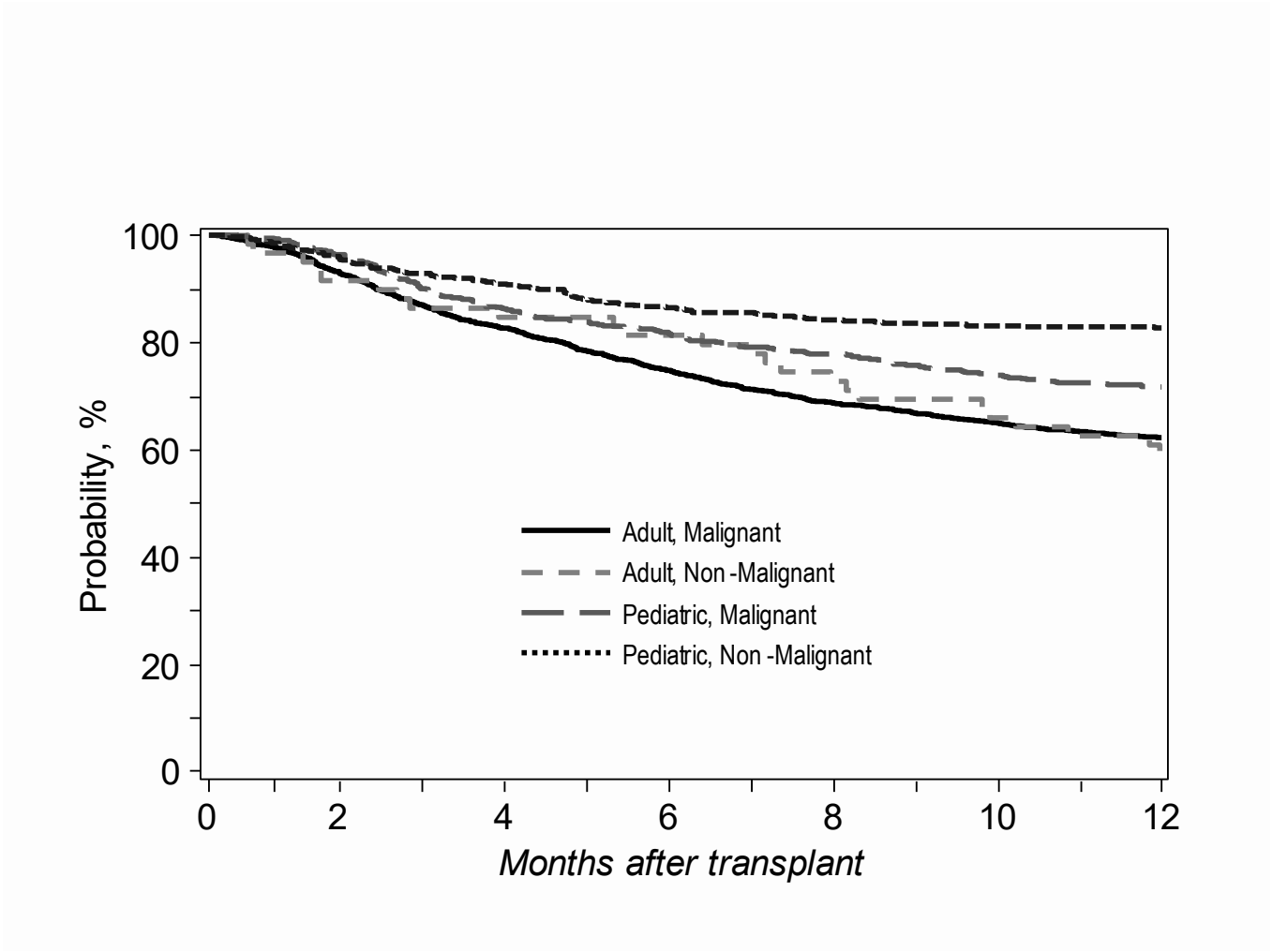

Figure 4-1: Survival after primary cord blood transplantation under Protocol 10-CBA between 10/20/2011 and 12/31/2021 by age-disease category

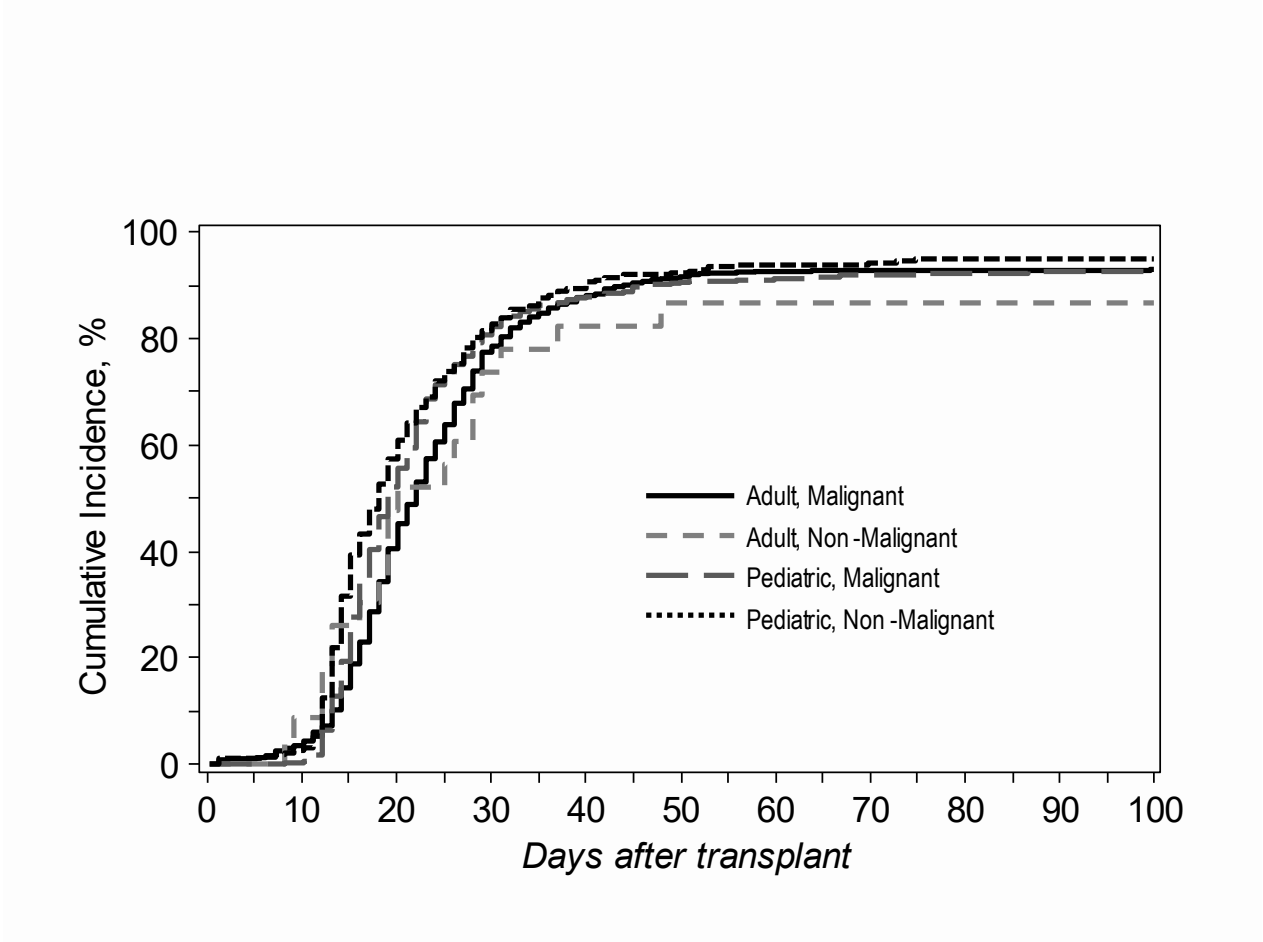

Figure 4-2: Neutrophil engraftment after primary cord blood transplantation under Protocol 10-CBA between 10/20/2011 and 12/31/2021 (myeloablative only) by age-disease category

Attachment 1

10-CBA Protocol
Version 10.3

Attachment 2
10-CBA
Investigator
Brochure
Revision 8