Advisory Council on Blood Stem Cell Transplantation (ACBSCT)

Program Update

Shelley Grant

September 13, 2013

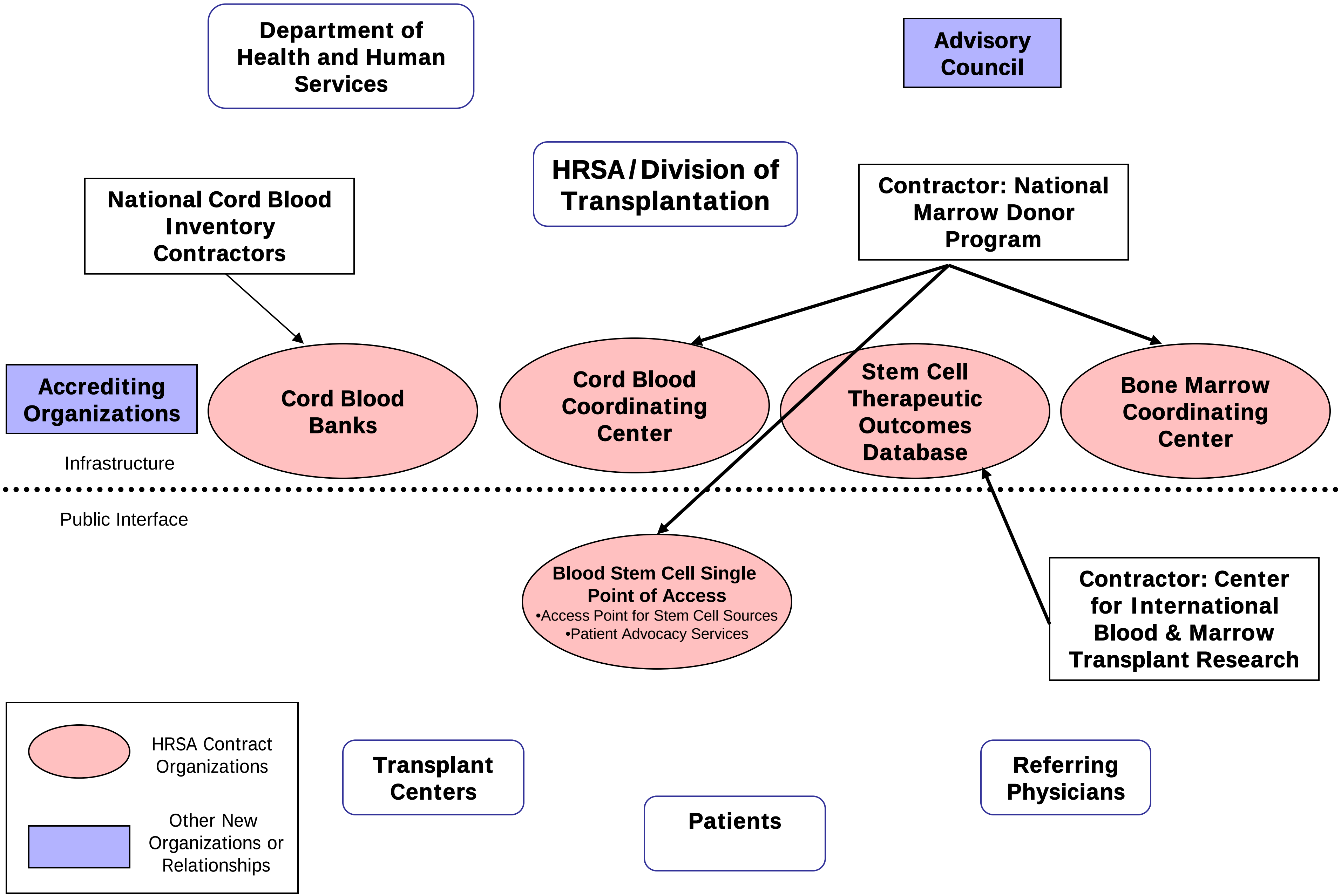
Presentation Overview

- Review legislative authority for C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory (NCBI)
- Provide status updates on the Program and NCBI
- Review appropriation levels for Program and the NCBI
- Review follow up items for HRSA’s Blood Stem Cell Transplantation Branch from previous meeting (May 2013)
- Share information from the Health Resources and Services Administration’s (HRSA) Request for Information (RFI)

C.W. Bill Young Cell Transplantation Program

- Authorized by Stem Cell Therapeutic and Research Act of 2005 (P.L. 109-129)
  - Reauthorized October, 2010 (P.L. 111-264)
- Goals are to increase:
  - Number of unrelated-donor transplants
  - Recruitment of potential marrow donors
  - Patient and donor advocacy services
  - Public and professional education re transplantation
  - Analysis and reporting of transplant outcomes data
- Opportunity to help more patients obtain transplants, other therapies, with blood stem cells

C.W. Bill Young Cell Transplantation Program (cont'd)



Department of Health and Human Services

Advisory Council

HRSA / Division of Transplantation

National Cord Blood Inventory Contractors

Contractor: National Marrow Donor Program

Accrediting Organizations

Cord Blood Banks

Cord Blood Coordinating Center

Stem Cell Therapeutic Outcomes Database

Bone Marrow Coordinating Center

Infrastructure

Public Interface

Blood Stem Cell Single Point of Access
- Access Point for Stem Cell Sources
- Patient Advocacy Services

Contractor: Center for International Blood & Marrow Transplant Research

⬭ HRSA Contract Organizations

▭ Other New Organizations or Relationships

Transplant Centers

Patients

Referring Physicians

Update on the C.W. Bill Young Cell Transplantation Program (Program)

- The Program continues to serve a growing number of patients in need of unrelated donor transplantation
  - As of September 30, 2012 (end FY 2012), the Program's registry included approximately 10.5 million potential adult donors
    - 28% from minority populations
  - In FY 2012, 508,716 adult potential donors were added to the Program's registry
    - 240,657 (47%) self-identified as belonging to racial/ethnic minority group

Update on the C.W. Bill Young Cell Transplantation Program (Program), cont'd

- The total number of cord blood units available through the Program in FY 2012 exceeded 163,000
- Transplants overall increased from 5,573 in FY 2011 to 5,833 in FY 2012 (a 4.7% increase)
- Transplants for minority patients increased from 955 in FY 2011 to 1,021 in FY 2012 (a 6.9% increase)
    - Transplants for minority patients represent 17.5% of the total number of transplants facilitated through the Program in FY 2012
    - Minority adult donor transplants grew from 542 in FY 2011 to 624 in FY 2012 (a 15.1% increase)

Update on the C.W. Bill Young Cell Transplantation Program (Program), cont'd

- Patient survival, one-year post-transplant, has increased significantly over the past ten years
- In FY 2013, the Program is on track to meet or exceed its performance goals
  - Increase the number of adult volunteer donors of minority race or ethnicity listed on the Program's registry to 2.85 million
  - Increase the number of blood stem cell transplants facilitated annually by the Program to 5,513
  - Increase the number of blood stem cell transplants facilitated annually by the Program for minority patients to 845

The National Cord Blood Inventory (NCBI)

NCBI Statistics

- 53,609 NCBI cord blood units available through the Program, as of September 30, 2012
  - Subsequent slide provides CBU demographics and contract targets
- 714 NCBI CBU distributed for transplant in FY 2012
  - 690 in FY 2011
- >27,000 CBU distributed for research by NCBI banks through end FY 2012
- The Fiscal Year 2013 NCBI appropriations supports adding approximately 7,800 cord blood units

Appropriation Levels by Fiscal Year	C.W. Bill Young Cell Transplantation Program	National Cord Blood Inventory (NCBI)
2011 President’s Budget	$26,544,000	$13,883,000
2011 Final	$23,374,000	$11,910,000
2012 President’s Budget	$26,544,000	$13,883,000
2012 Final	$23,374,000	$11,887,000
2013 President’s Budget	$23,330,000	$11,887,000
2013 Final	$21,877,000	$11,147,000
2014 President’s Budget	$23,330,000	$11,887,000

Follow up items from May 2013 ACBSCT meeting

- Provide the Advisory Council with data on transplants by indication
- Work with Realizing the Potential of Cord Blood Work Group and the Scientific Factors Necessary to Define Cord Blood Units as High-Quality Units Work Group to ensure that HRSA considers all the possibilities for addressing cord blood bank financing
- Provide the Advisory Council with information regarding progress on educational/outreach efforts to the sickle cell disease (SCD) patient and provider community

Transplants by Indication

- HRSA provided the Advisory Council members with a list of transplant indications in its packet of information
- Transplant data by indication are searchable via HRSA's .gov Website: http://bloodcell.transplant.hrsa.gov/research/transplant_data/us_tx_data/data_by_disease/national.aspx. The tool allows users to extract data by disease, age, race, cell sources and more.
- Source: http://bethematch.org/Patient/Disease_and_Treatment/About_Your_Disease/Learning_More_about_Your_Disease.aspx

Cord Blood Financing

- Share information from HRSA’s Request for Information with the Advisory Council’s Work Groups on Realizing the Potential of Cord Blood and the Scientific Factors Necessary to Define Cord Blood Units as High-Quality Units to ensure that HRSA is considering all the possibilities for addressing cord blood bank financing

Outreach effort to the SCD patient and physician community

- Met with Dr. Donnell Ivy, Medical Officer, Genetics Services Branch, Division of Services for Children with Special Needs, Maternal and Child Health Bureau, Health Resources and Services Administration
  - Discussed how to best coordinate on common areas of interest
- Other activities:
  - Referring Physician Education
    - Included SCD in clinical guidelines
  - Patient Education
  - Exhibited and provided educational materials at the Sickle Cell Association of America, September 2012

Outreach effort to the SCD patient and physician community (cont'd)

- Other activities (cont'd):
  - Exhibiting and providing educational materials at the Sickle Cell Association of America 41st Anniversary Convention in Baltimore, MD, September 24-27, 2013
  - Promoted SCD awareness during African American Bone Marrow Awareness Month (July 2012 and 2013)
  - Reviewed and updated the family information brochure for the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) protocol 0601, The SCURT Study (Evaluating the Safety and Effectiveness of Bone Marrow Transplant in Children With Sickle Cell Disease), June 2012

Request for Information (RFI) for HRSA's National Cord Blood Inventory

- HRSA released the RFI on July 8, 2013 in Fedbizopps
- The current due date is September 27, 2013
- The purpose of the RFI is:
  - To provide the public with a description of, and an opportunity to submit comments on, HRSA's current approach to obtaining cord blood units for the National Cord Blood Inventory.
  - To provide the public with an opportunity to propose demonstration and outreach projects that HRSA may consider implementing through new or existing contract(s) as a means of increasing donations of cord blood units (CBU) from diverse populations.
  - To identify additional organizations interested in and capable of providing cord blood units to the National Cord Blood Inventory.

RFI (cont'd)

- HRSA has received approximately ten responses to date
- The respondents represent the following types of organizations: public and private cord blood banks, HRSA contractors, private cord blood bank foundations, private information technology companies
- Some of the themes received to date:
    - Continue funding of Caucasian cord blood units
    - Increase total nucleated cell count (TNC) for qualification of NCBI funding
    - Provide funding for maternal sample typing projects

RFI (cont'd)

- Themes (cont'd):
  - Need for Extra support with the Food and Drug Administration's licensure process and ISBT 128 requirements
    - ISBT 128 is a system for identification, labeling, and processing of human blood, tissue, and cellular therapy products using an internationally standardized system
  - Need for more educational outreach and a call center for all uses of umbilical cord blood (i.e., public donation, family banking, research, discard as medical waste)
  - Received capability statements for more diverse cord blood collections and single point of access infrastructure for searching for cord blood units

RFI (cont'd)

- Themes (cont'd):
  - Simplify the cord blood donor consent process
  - Provide up-front financial support to cord blood banks
  - Increase partnerships between public and private cord blood banks
  - Provide more financial reimbursement for NCBI cord blood units in general and specifically for African American/Black and Asian/Pacific Islander ethnicity
  - Integrate the remote kit collection program with an private information technology system to increase diverse cord blood unit collections

RFI (cont'd)

- There is still time to respond:
- **DEADLINE:** All responses must be received no later than 4 p.m. EST on September 27, 2013, at the address listed below.
- **ADDRESSES:** HHS/HRSA, Kimberly Lewis, 5600 Fishers Lane, Room 13A-19, Rockville, Maryland 20857, KBLewis@hrsa.gov, 301.443.4540.
- https://www.fbo.gov/index?s=opportunity&mode=form&id=4a25be8a3cb7d01a3f5a5f1df281c218&tab=core&_cview=1

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Questions?