



HRSA Update to the Advisory Council on Blood Stem Cell Transplantation

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Presentation Overview

- Review legislative authority for Stem Cell Therapeutic and Research Reauthorization Act of 2010
- Update on C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory (NCBI)
- Review Appropriation level for Program and NCBI
- Discuss importance of balancing Program and NCBI funds
- Share statistics for Program and NCBI
- Provide status of Notice of Public Rule Making (NPRM) published in October 2013
- Share information regarding letter received by HRSA regarding NCBI and defining high quality cord blood units
- Introduce the Blood Stem Cell Transplantation Team

C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory (NCBI)

- 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
 - Provide funding to cord blood banks to collect, process, store and make available high-quality, diverse umbilical cord blood units (CBU)
 - Target the collection of at least 150,000 new units
 - Make cord blood units that are not appropriate for transplantation available for research
- Opportunity to help more patients obtain transplants, other therapies, with blood stem cells

Update on the C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory

- The HRSA Programs continue to serve a growing number of patients in need of unrelated donor transplantation
 - As of September 30, 2013 (end of FY 2013), the Program's registry included approximately 11.2 million adult donors
 - More than 3.05 million (27 percent) self-identified as a racial/ethnic minority
 - In FY 2013, nearly 400,000 adult volunteer donors were added to the Program's registry
 - 205,607 (52%) self-identified as belonging to an under-represented racial/ethnic minority group

Update on the C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory (NCBI), cont'd

- The total number of transplants facilitated in FY 2013 was 6,283 (compared to 5,832 in FY 2012 – a 7.7% increase)
- The total number of domestic transplants was 4,866 in FY 2013 (compared to 4,427 in FY 2012 - a 9.7% increase)
- The total number of cord blood transplants (NCBI and non-NCBI) was 1,102 (compared to 1, 191 in FY 2012 –a decrease of 7.4%)
- The total number of cord blood units (NCBI and non-NCBI) shipped in FY 2013 was 1,575 (compared to 1, 637 in FY 2012 –a decrease of 3.8%)

Update on the C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory (NCBI), cont'd

- The total number of cord blood units (NCBI and non-NCBI) available through the Program in FY 2013 exceeded 243,000 (including New York Cord Blood Bank)
- 63,960 NCBI cord blood units available through the Program, as of September 30, 2013
- 714 NCBI CBU distributed for transplant in FY 2013
 - 714 in FY 2012

Appropriation Level by Fiscal Year	C.W. Bill Young Cell Transplantation Program	National Cord Blood Inventory (NCBI)
2013 Final	\$21,877,000	\$11,147,000
2014 Final	\$22,154,000	\$11,238,000
2015 President's Budget	\$22,109,000	\$11,266,000

Balancing the use of Program and NCBI funding

- Balancing the use of Program and NCBI funds is important as Congress expects all authorized functions of the Program and NCBI to be successful
- Working with Cord Blood Coordinating Center (CBCC) and cord blood banks to develop strategic priorities to support more robust cord blood initiatives
- Providing financial support for FY 2015 (through CBCC) to select NCBI banks to make cord blood units more rapidly available through the Program
- Conducting cord blood bank site visits to better understand the needs of the banks (particularly with decreased cord blood utilization)
- Competition for new banks to join the NCBI
 - None in FY 2014
 - FY 2015 competition, to be determined

Publically available information on HRSA's Notice of Proposed Rule Making (NPRM)

- Released on October 2, 2013
- Purpose – To seek public comment on the proposed change in the definition of “human organ” in section 301 of the National Organ and Transplant Act of 1984, as amended, (NOTA) to explicitly incorporate hematopoietic stem cells (HSCs) within peripheral blood in the definition of “bone marrow.” This would clarify that the prohibition on transfers of human organs for valuable consideration applies to HSCs regardless of whether they were recovered directly from bone marrow (by aspiration) or from peripheral blood (by apheresis). This amendment will also conform section 301 to the provisions of the Stem Cell Research and Therapeutic Act of 2005, as amended.

Publically available information on HRSA's NPRM (cont'd)

- Deadline: December 2, 2013
- Comments received: 533
- Next steps: pending

Question received after last Advisory Council Meeting in May 2014

- HRSA's Administrator, Dr. Mary Wakefield, received a letter from an interested party asking whether HRSA's information technology systems could support providing transplant physicians with any information that a cord blood bank felt was important prior to cord blood unit (cbu) selection
 - The answer is yes. HRSA's contractor has a robust computer system that currently allows for cord blood banks to enter, via a free text format, relevant information on its cord blood units
- The writer also asked if HRSA had mechanisms in place to continually evaluate, and change, if necessary, the definition of a high quality cbu
 - The answer is yes... through the work of this Advisory Council



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