



Program Report 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 2015
- Review Appropriation levels for C.W. Bill Young Cell Transplantation Program (Program) and National Cord Blood Inventory (NCBI)
- Update on Program and NCBI
- Share current priorities for The Division of Transplantation's (DoT) - Blood Stem Cell Transplantation Branch

C.W. Bill Young Cell Transplantation Program (Program and NCBI)

- Authorized by Stem Cell Therapeutic and Research Act of 2005 (P.L. 109-129)
 - Reauthorized October, 2010 (P.L. 111-264)
 - Reauthorized December, 2015 (P.L. 114-104)
- Program Goals are to increase:
 - Number of unrelated-donor transplants
 - Recruitment of potential marrow donors
 - Patient and donor advocacy services
 - Public and professional education regarding transplantation
 - Analysis and reporting of transplant outcomes data

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

- **NCBI Goals are to:**
 - Provide funding to cord blood banks to make available high-quality, diverse umbilical cord blood units
 - Add at least 150,000 new cord blood units
 - Make cord blood units, not appropriate for transplantation, available for research

Significant Changes in New Legislative Authority

The HHS Secretary, in consultation with the National Institutes of Health, Food and Drug Administration, HRSA, the Advisory Council, and other stakeholders shall review the state of the science of using adult cells and birthing tissue to develop new therapies for patients, for the purpose of inclusion in the C.W. Bill Young Cell Transplantation Program

Significant Changes in New Legislative Authority (cont'd)

- Consideration of the Best Science
 - The Secretary shall take into consideration the best scientific information available in order to maximize the number of cord blood units available for transplant when entering or extending period of funding under the NCBI contracts

Significant Changes in New Legislative Authority (cont'd)

- No later than one year after the date of enactment (December, 2015), the Secretary of Health and Human Services shall issue determinations with respect to inclusion of peripheral blood stem cells and umbilical cord blood in the definition of human organs

Appropriation Level by Fiscal Year	C.W. Bill Young Cell Transplantation Program	National Cord Blood Inventory (NCBI)
2014 Final	\$22,154,000	\$11,238,000
2015 Final	\$22,109,000	\$11,266,000
2016 President's Budget	\$22,109,000	\$11,266,000
2017 President's Budget	\$22,109,000	\$11,266,000

Current Status of the Program

- The Program serves a growing number of patients in need of unrelated donor transplantation
 - As of September 30, 2015 (end of FY 2015), the Program's registry included approximately 13.6 million adult donors
 - More than 3.40 million (approx. 25%) self-identified as belonging to a specific racial/ethnic minority

Current Status of the Program (cont'd)

- The total number of cord blood units (CBU) available through the Program in FY 2015 exceeded 225,000 (NCBI and non-NCBI CBU)
- The total number of transplants facilitated in FY 2015 was 6,373 (compared to 6,253 in FY 2014 - a 1.9% increase)
- The total number of domestic transplants was 5,078 in FY 2015 (compared to 4,892 in FY 2014 - a 3.8% increase)
- The total number of cord blood transplants (NCBI and non-NCBI) was 992 in FY 2015 (compared to 960 in FY 2014 - a 3.3% increase)
- The total number of CBU (NCBI and non-NCBI) shipped in FY 2015 was 1,393 (compared to 1,359 in FY 2014 - an increase of 2.5%)

Establishing Funding Priorities

- HRSA may consider funding:
 - Cord blood banks (CBB) collecting only, or a high percentage of, minority units (particularly from Asians and Black/African Americans)
 - CBB with proven experience in meeting established goals
 - CBB that have received FDA licensure
 - CBB that offer the government significant discounts/best value
 - CBB that are financially stable
 - CBB that are able to expand cord blood collections and deliver more CBU annually
- Identification of special projects that will increase the diversity of CBU added to the Program is a priority

Other Priorities for DoT's Blood Stem Cell Transplantation Program

- Assess data regarding the number of unrelated blood stem cell transplants facilitated through the Program
 - Broken down by graft source
 - Overall and domestic
- Understand the role of haplo-identical transplants, and other therapies, and their:
 - Impact on cord blood utilization
 - Impact on access to transplantation by patients who are not adequately served

Other Priorities for DoT's Blood Stem Cell Transplantation Program (cont'd)

- Continue to leverage available data and resources to drive policy decisions.
 - Hiring a new biostatistician
- Ensure that appropriate questions are being asked of the Advisory Council to raise awareness about this evolving field regarding:
 - Scientific factors for defining a high quality cord blood unit
 - Role of cellular therapy (using adult donors and cord blood) in the C.W. Bill Young Cell Transplantation Program
 - Increasing access to transplantation
 - Single Point of Access for adult donors and cord blood units (alternative approaches for consideration)



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