Section 1: Introduction

1.0 Overview of the C.W. Bill Young Cell Transplantation Program

Every year, thousands of men, women and children are diagnosed with life-threatening diseases such as leukemia and lymphoma. Many of them will die unless they get a bone marrow, peripheral blood stem cell or cord blood transplant from a genetically matched donor. While some will find a donor within their family, most do not and need help to find an unrelated bone marrow, peripheral blood stem cell or cord blood donor. They also need their physicians to know and use the latest, most effective transplant practices. This knowledge comes from analyzing the outcomes of many transplants from centers around the world. All of these patients depend on the knowledge and services provided by the C.W. Bill Young Cell Transplantation Program. Additional general information about hematopoietic stem cell transplantation (HCT) can be found in section 2.4.4.

This Biennial Report describes the activities of the C.W. Bill Young Transplantation Program and its predecessor, the National Bone Marrow Donor Registry, for the period January 2008 through December 2009. It is an overview of the operation of this extensive network of medical organizations that provides patients and providers with the hematopoietic stem cells and information they need to combat life-threatening diseases.

1.1 History

In December 2005, the Stem Cell Therapeutic and Research Act of 2005 (Public Law 109-129) established the C.W. Bill Young Cell Transplantation Program (the Program). The Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) is responsible for overseeing this Program.

The C.W. Bill Young Cell Transplantation Program (the Program) is the successor to the National Bone Marrow Donor Registry (the Registry). The Registry was begun in 1987 through a grant from the U.S. Navy. In 1990, the Registry was formally established under HHS, with oversight initially by the National Heart, Lung, and Blood Institute (NHLBI), and since 1994 under HRSA. The first transplant under the Registry's auspices took place in 1987. While some of the activities of the Program are similar to those of the Registry, the Program has the added responsibility of collecting, analyzing and reporting on outcomes for all allogeneic transplants (those from a donor other than the patient him/herself) and on other therapeutic uses of blood stem cells. The Program was designed to help patients who need a transplant from an unrelated adult marrow or peripheral blood stem cell (PBSC) donor or cord blood unit (CBU) by:

- Making information about bone marrow and cord blood transplantation available to patients, families, health care professionals and the public
- Providing efficient, effective processes for identifying unrelated matched marrow and PBSC donors and cord blood units through one electronic system
- Increasing the numbers of unrelated marrow donors and cord blood units that are available
- Expanding research to improve patient transplant outcomes

1.2 Legislation and Components

The Stem Cell Therapeutic and Research Act of 2005 covers several components:

- The C.W. Bill Young Cell Transplantation Program. The Program's infrastructure is composed of:
    - The Office of Patient Advocacy and Single Point of Access (OPA/SPA),

  - The Bone Marrow Coordinating Center (BMCC),
  - The Cord Blood Coordinating Center (CBCC), and
  - The Stem Cell Therapeutic Outcomes Database (SCTOD).
- The Program expands on what the National Bone Marrow Donor Registry was doing to increase the number of marrow donors and cord blood units.
- The National Cord Blood Inventory (NCBI). NCBI umbilical cord blood banks will collect and store 150,000 new, high-quality umbilical cord blood units that will be made available to patients. The NCBI banks will also make some CBUs that do not meet the criteria for transplant available to researchers.
- The Stem Cell Therapeutic and Research Act also authorized establishment of an Advisory Council on Blood Stem Cell Transplantation (Advisory Council). This council makes recommendations on issues about the Program to the Secretary of Health and Human Services and HRSA.

Figure 1 shows how the Program is organized.

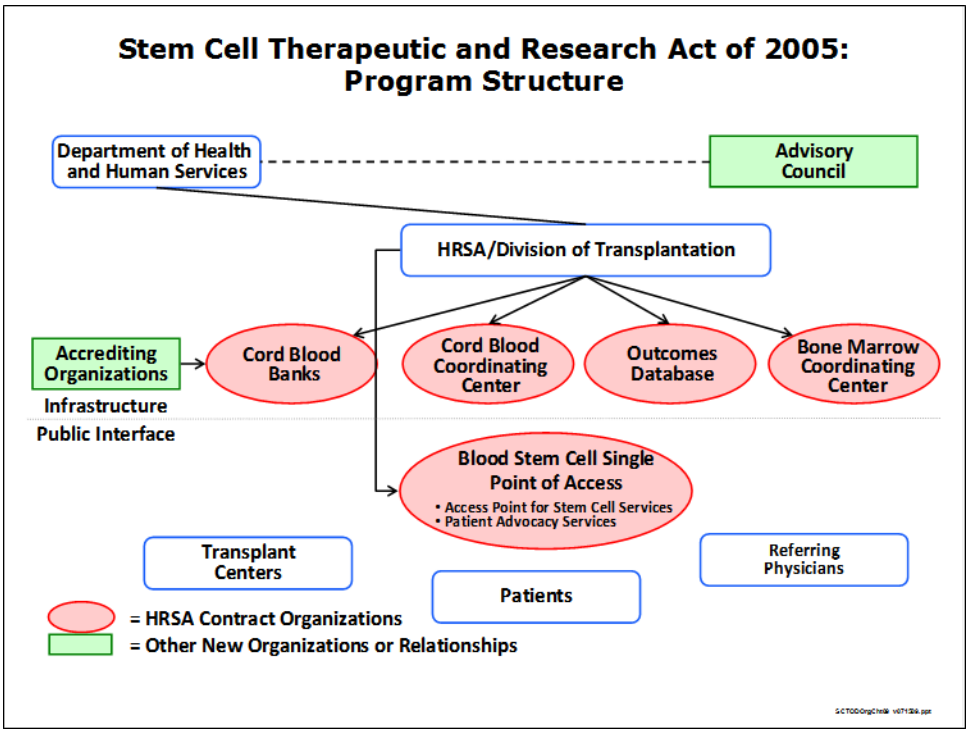

Figure 1. Organizational Structure for the Program under the Stem Cell Therapeutic and Research Act of 2005

1.3 Responsibilities

The four Program components work together to:

- Operate an electronic system for identifying, matching and facilitating the distribution of blood stem cells
- Allow transplant physicians, health care professionals and patients to search electronically for available cord blood units and unrelated adult PBSC or marrow donors
- Educate and reach out to the public to increase the numbers of volunteer adult PBSC, bone marrow and cord blood donations to ensure genetic diversity
- Promote the availability of cord blood units as a transplant option
- Analyze transplant trends and support research to help more transplant recipients live longer, healthier lives

1.4 Program Contractors

The **National Marrow Donor Program® (NMDP)** operates three of the Program components: the Office of Patient Advocacy and Single Point of Access (OPA/SPA), the Bone Marrow Coordinating Center (BMCC) and the Cord Blood Coordinating Center (CBCC).

NMDP is a nonprofit organization based in Minneapolis, Minnesota, which operated the National Bone Marrow Donor Registry under a series of contracts from the Federal Government. The NMDP is dedicated to creating opportunities for all patients to receive a bone marrow, PBSC or umbilical cord blood transplant from an unrelated donor when they need it. The Registry has grown to include more than eight million donors and more than 120,000 CBUs. It is the largest and most racially and ethnically diverse registry of its kind in the world. Medical advances are making marrow, peripheral blood and cord blood transplants available to more patients all the time. Since the first unrelated transplant was facilitated in 1987, NMDP has facilitated more than 38,000 transplants. It now facilitates more than 4,800 transplants a year, analyzes the outcomes of the donation process for unrelated donors, and maintains a repository of donor-recipient blood samples for a large proportion of these transplants.

The **Center for International Blood and Marrow Transplant Research (CIBMTR)** began as the International Bone Marrow Transplant Registry (IBMTR) at the Medical College of Wisconsin in 1972. By 2004, the IBMTR had grown to become a voluntary network of more than 400 hematopoietic stem cell transplant (HCT) centers in 47 countries that shared data and conducted scientific studies to improve transplant outcomes. The physicians and scientists of IBMTR had generated more than 200 scientific peer-reviewed publications by 2004. At that time, the CIBMTR was created through an affiliation between IBMTR and the research arm of NMDP.

The CIBMTR operates the fourth component of the Program: the Stem Cell Therapeutic Outcomes Database (SCTOD). CIBMTR analyzes the outcomes of all reported transplants, including since 2006, those transplants using unrelated donors facilitated by NMDP. The affiliation between IBMTR and NMDP-Research specified that the two organizations will conduct all of their HCT-related research activities together. This has led to coordinated data collection, management and analytic procedures. It takes advantage of the strengths of each of the two organizations to substantially enhance the resources that are available to the transplant community.

The IBMTR brought a strong track record of statistical and clinical HCT expertise and an international network of data on autologous, related and unrelated donor transplants. NMDP-Research brought expertise in HLA[1] matching, bioinformatics, computer systems and repository maintenance. Integrating these operations allows more efficient data management, enhances electronic data collection capabilities and gives opportunities to link clinical data with biologic specimens.

1 Human leukocyte antigen (HLA) typing is used to match patients and donors. HLAs are proteins—or markers—found on most cells in your body. The immune system uses these markers to recognize which cells belong in your body and which do not. A close match between a patient's and donor's HLA can reduce the risk that the patient's immune cells will attack the donor cells or that the donor's immune cells will attack the patient's body after the transplant.