



**Written Testimony of Dr. Jeffrey W. Chell
before the House Committee on Energy and Commerce
Hearing on “Examining Public Health Legislation:
H.R. 2820, H.R. 1344, and H.R. 1462”
June 25, 2015**

Summary of Key Points

- The NMDP/Be The Match appreciates the continuing bipartisan support to reauthorize the C.W. Bill Young Cell Transplantation Program and the National Cord Blood Inventory. It is critically important to reauthorize these programs before their authorizations expire at the end of September 2015.
- Since the late Congressman Bill Young was first inspired by 11-year-old Brandy Bly who did not survive her battle with leukemia and he testified before the Commerce Committee in 1987, significant progress has been made to provide access to transplant for all Americans in need of a life-saving bone marrow or cord blood transplant.
- Today, the NMDP/Be The Match is the global leader in helping patients seeking a cure for one of the more than 70 different diseases or conditions, including blood cancers and sickle cell disease. Cellular therapy is their best hope for a cure.
- The Be The Match Registry has grown to include nearly 12.5 million donors and more than 209,000 cord blood units and through international relationships, we have access to more than 24.5 million potential donors and 622,000 cord blood units worldwide.
- Between 1988 and 2005, the one-year survival rate for these patients has increased from just over 40 percent to 70 percent. Access to multiple cell sources allows us to find the best match possible for patients.
- In 2014, we facilitated more than 1,200 unrelated transplants for patients 18 or younger using bone marrow or cord blood. Current pediatric research focuses not only on malignancies, but also on curing non-malignant diseases such certain metabolic diseases and hemaglobinopathies like sickle cell disease and thalassemia.
- The calculated need for unrelated transplant has increased by 25 percent since 2005. Much of the reason is due to the expansion in the number of indications for transplant.
- The number of transplants for minority patients has increased from 253 in 2000 to 990 in 2014.
- The non-match barriers to access and care have a profound affect on our ability to make transplant therapies accessible to all.

Good morning Mr. Chairman, and other distinguished members of the Subcommittee. My name is Dr. Jeffrey Chell and I am the Chief Executive Office of the National Marrow Donor Program (NMDP)/Be The Match. We operate the C.W. Bill Young Cell Transplantation Program (Program) through four competitively bid contracts with the Health Resources and Services Administration (HRSA). These contracts include the Single Point of Access and Office of Patient Advocacy, the Bone Marrow Coordinating Center, and the Cord Blood Coordinating Center. Collectively, these contracts allow NMDP/Be The Match to operate the national Be The Match Registry and provide life-saving blood and marrow transplants using individual adult donors and umbilical cord blood units. And with the Medical College of Wisconsin, NMDP/Be The Match also holds the contract for the Stem Cell Therapeutic Outcomes Database, known as the Center for International Blood and Marrow Transplant Research (CIBMTR) of which I am the executive director.

To begin, I would like to thank Chairman Pitts, Ranking Member Green, and all of the Members of the Health Subcommittee for inviting me to speak with you today. On behalf of 565 Network partner organizations and everyone at the NMDP/Be The Match, we also want to thank you for maintaining the Congressional commitment to patients fighting blood cancers and other disorders whose only hope for a cure is a bone marrow or cord blood transplant.

I would also like to thank Congressman Chris Smith, Congresswoman Doris Matsui, Congressman David Jolly, and Congressman Chaka Fattah for their leadership in introducing H.R. 2820 to reauthorize the Program, as well as the National Cord Blood Inventory (NCBI) grant program. These programs are examples of how the Congress can inspire innovation to bring cures to patients across America.

Who We Are

As I testify before you today, I cannot help but be reminded of another hearing that took place in 1987. On that day, the late Congressman Bill Young called on the Congress to establish a national registry where men, women, and children with leukemia and other fatal blood disorders could find an unrelated donor to save their lives. He was joined by Dr. Robert Graves, whose daughter received the first unrelated bone marrow transplant for leukemia, and Navy Admiral Bud Zumwalt. The Congress heard that call and established the national registry. First housed in the Department of the Navy, it found its permanent home as one of the nation's premier public health programs at HRSA.

Congressman Young's quest was inspired by a child, 11-year-old Brandy Bly, and her family racing to save her life. Unfortunately, no one in her family was a suitable match and she did not survive. It was the simple statement from her physician – "Wouldn't it be great if there were a way that doctors could search for adults willing to donate their bone marrow?" – that led to the national registry.

Since that first hearing in 1987, we have made great progress. Today, the NMDP/Be The Match is the global leader in providing a cure to patients with life-threatening blood and marrow cancers such as leukemia and lymphoma, as well as other diseases. Through the contract with HRSA, we manage the world's largest registry—the Be The Match Registry—of potential marrow donors and umbilical cord blood units, connect patients to their donor match for a life-saving marrow or umbilical cord blood transplant, educate health care professionals, and conduct research so more lives can be saved. Today, children like Brandy have a much better chance to find that life-saving match and ultimately a cure.

But, the C.W. Bill Young Program as it has come to be known, is more than the national registry. Through the Program, the NMDP/Be The Match also operates the Office of Patient Advocacy. The Office of Patient Advocacy assists patients and their families in navigating the complexities of health insurance and helps them overcome logistical, psychosocial and informational barriers throughout the transplant continuum. We also work closely with donor and collection centers through the Bone Marrow Coordinating Center contract to recruit and retain volunteer potential donors, produce a comprehensive plan for donor retention, and plan for increasing operational efficiencies. We similarly provide financial and educational support to public cord blood banks as the manager of the Cord Blood Coordinating Center and provide guidance to HRSA in the administration of the National Cord Blood Inventory program to determine optimal composition of cord blood inventory. And, we partner with the Medical College of Wisconsin to operate the Stem Cell

Therapeutic Outcomes Database, which facilitates research to improve patient outcomes and find new and exciting ways bone marrow and umbilical cord blood can be used to save lives.

The Success of the Program

During the past 28 years, the NMDP/Be The Match has been honored to serve as the steward of this critical national resource. Today, the Be The Match Registry serves as the single point of access for both umbilical cord blood units and adult volunteer donors. This single point of access assures that these physicians will have access to any potential donor or umbilical cord blood unit regardless of where located across the globe in order to perform adult stem cell transplants that can cure more than 70 different diseases or conditions, including blood cancers and sickle cell disease. The Be The Match Registry has grown to include nearly 12.5 million donors and more than 209,000 cord blood units. Through international relationships, the NMDP/Be The Match has access to more than 24.5 million potential donors and 622,000 cord blood units worldwide.

For patients battling these fatal cancers and other blood disorders, the NMDP/Be The Match offers support and education to help them live healthy lives after transplant. We provide patient services, caregiver support, and financial support through the Be The Match Foundation. We also work closely with transplant physicians throughout the country by developing and improving upon post-transplant guidelines to improve survival rates.

Our focus is on patients for whom cellular therapy is the best hope for cure of their diseases and is often the only therapy available with an intent to cure. Today, we are able to treat patients with cancers and pre-cancers, such as leukemia, Myelodysplasia, and lymphomas; bone marrow failure disorders, such as aplastic anemia and immunodeficiency syndromes; and genetic diseases, such as sickle cell disease. To treat these diseases, we infuse bone marrow, peripheral blood stem cells, or cord blood cells into a patient after having eliminated his/her current bone marrow. These new cells restore the patient's ability to make blood cells or provide a new immune system to attack cancer cells. Finding the best match possible is important because if donor stem cells are not the same HLA type as the recipient they will recognize the recipient as being different and attack, leading to rejection.

Because we collect data on all transplants, we have been able to improve patient outcomes and reduce rejection. Between 1988 and 2005, the one-year survival rate for these patients has increased from just over 40 percent to 70 percent. Access to multiple cell sources allows us to find the best match possible for patients. Initially focused only on bone marrow, the Program today also allows physicians to select peripheral blood stem cells and cord blood, as well as bone marrow, as the source of the adult stem cells used in transplant.

We are especially proud of the work we have done to help children in need of a transplant. In 2014, the NMDP/Be The Match facilitated more than 1,200 unrelated

transplants for patients 18 or younger using bone marrow or cord blood. Our current research focuses not only on malignancies, but also on curing non-malignant diseases such as certain metabolic diseases and hemoglobinopathies like sickle cell disease and thalassemia. These blood disorders can be fatal if left untreated. Prior to transplantation therapy, children with these diseases would often die prematurely.

We are also learning more about how to improve outcomes for children fighting blood cancers. By having access to all three sources of adult stem cells, physicians can select the best source to meet their young patients' needs. For example, most physicians prefer a fully matched bone marrow graft if available for all patients, including children. In certain instances, umbilical cord blood is used, especially if there is no fully matched adult donor. In this way, umbilical cord blood has significantly extended the opportunity for all patients who otherwise would not have found an acceptable adult match. Through the CIBMTR, researchers throughout the world are finding new and exciting ways that bone marrow and cord blood transplants can help children fight life-threatening diseases.

More Needs To Be Done

However, more needs to be done. The need for transplants is increasing, especially among older Americans. The calculated need for unrelated transplant has increased by 25 percent since 2005. Much of the reason is due to the expansion in the number of indications for transplant, as well as advances that allow older Americans to be

candidates for transplants. Transplants for patients 51-64 years old are growing faster than other age groups. NMDP/Be The Match facilitated transplants have grown by 200 percent overall and 250 percent for minorities since 2006.

While we have made significant improvements in transplants for racial and ethnic minority patients, there too more work is needed. The number of transplants for minority patients has increased from 253 in 2000 to 990 in 2014. We continue our efforts to expand the diversity of the national adult volunteer donor registry and 46 percent of cord blood units on the registry are from a minority donor. During the last 5 years cord blood has been the product source for about 21 percent of all transplants and 37 percent of minority patients who received a transplant relied upon cord blood.

Federal funding remains critical to continuing to provide access to transplantation. We need to continue to recruit new potential donors both to improve access for minority patients and to renew the current list of donors with younger donors. Grafts from younger donors have shown improved clinical outcomes. For every one million dollars allocated by the Congress, the Program can add 10,000 adult volunteer donors or 750 cord blood units to the national registry. Preserving these funds through the reauthorization of the Program allows it to continue to improve the chances of every American needing a transplant to find a match and provides the critical infrastructure that allows NCBI cord blood units to be used to save lives.

Even though the NMDP/Be The Match has improved the ability of those needing a transplant to find a match, there are other barriers that continue to make access difficult. These non-match barriers to access and care have a profound affect on our ability to make transplant therapies accessible to all. Language, literacy, finances, insurance, geography, lack of knowledge, and predisposition by general hematologists and oncologists towards non-transplant therapies all have an impact. The NMDP/Be The Match continues to work with patients, physicians, community leaders, and others to address these problems as well.

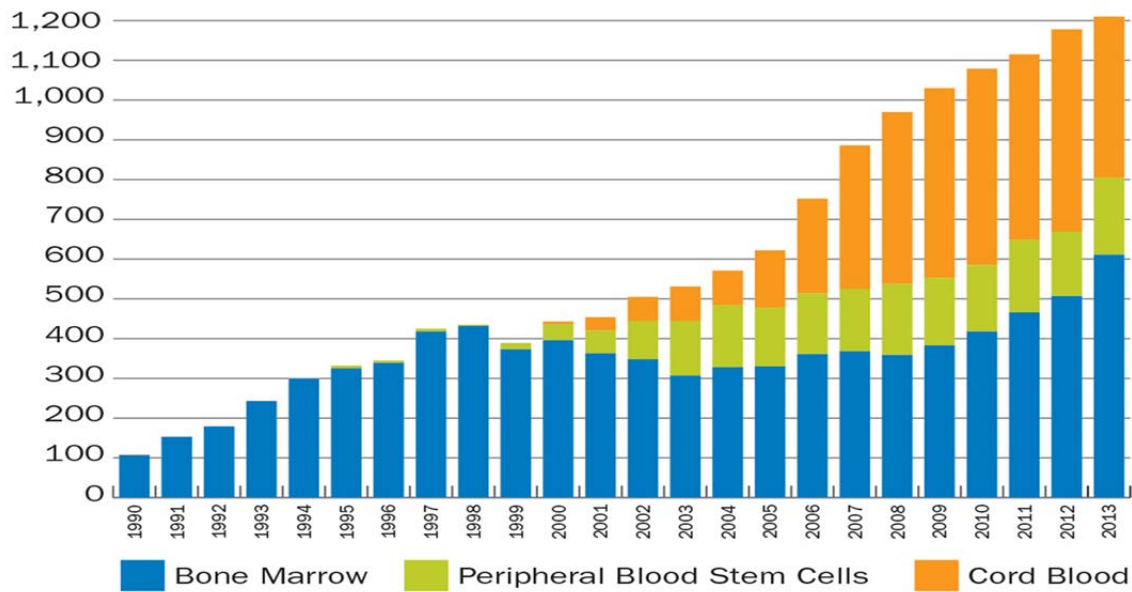
Conclusion

The ongoing Congressional commitment has made these advances possible and turned the tragic loss of 11-year-old Brandy Bly into hope for tens of thousands of Americans. One of those Americans is Hadley Mercer. When she was just six months old, Hadley was diagnosed with acute myeloid leukemia (AML). After two rounds of chemotherapy, her parents began to consider a bone marrow transplant as an alternative treatment option for Hadley. They consulted various physicians, who all agreed that having a bone marrow transplant would be her best chance of survival. The family held a bone marrow drive and registered more than 1,000 people in five hours. A few months later, Hadley was matched with a young man in his twenties. Now almost two, Hadley is alive because of her “donor angel”. She is also alive because of your continued support for the C.W. Bill Young Cell Transplantation Program.

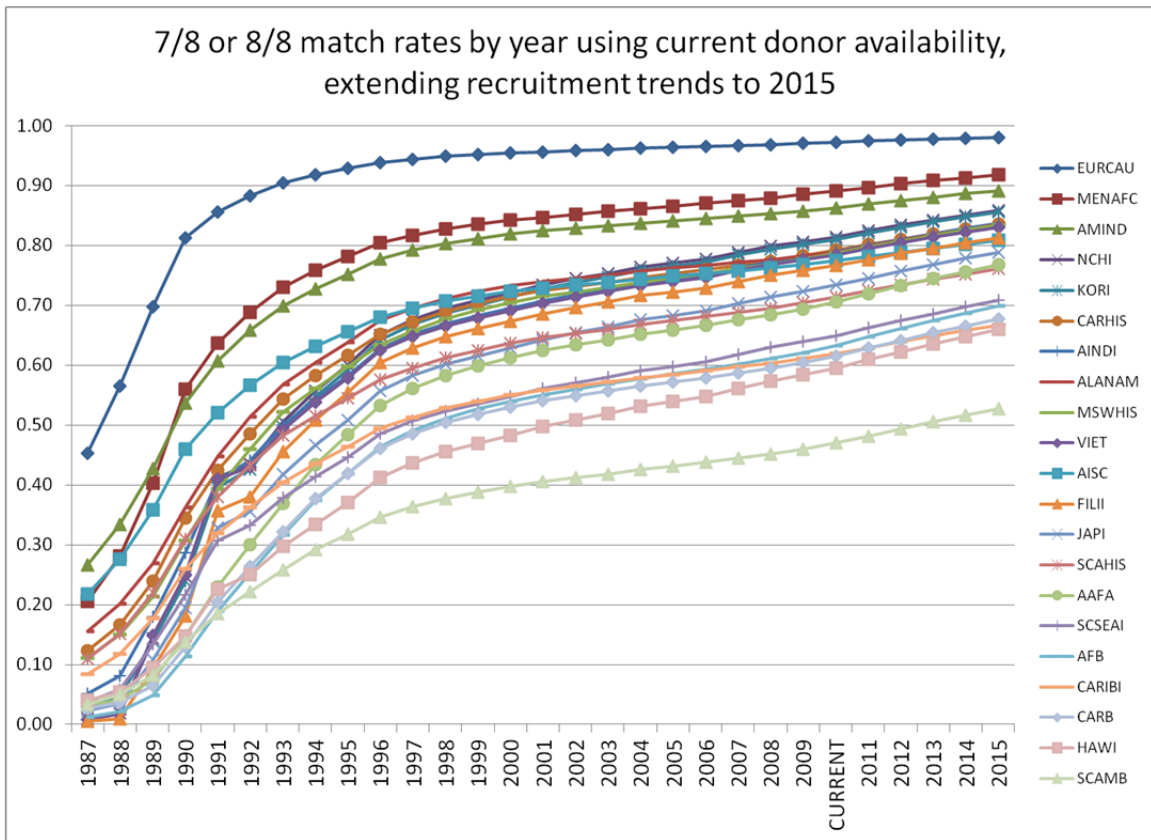
The NMDP/Be The Match has never forgotten the importance of the physician's simple statement that inspired Congressman Young and every day we are inspired by the people we meet, young and old, who are seeking to find a match and undergo a transplant.

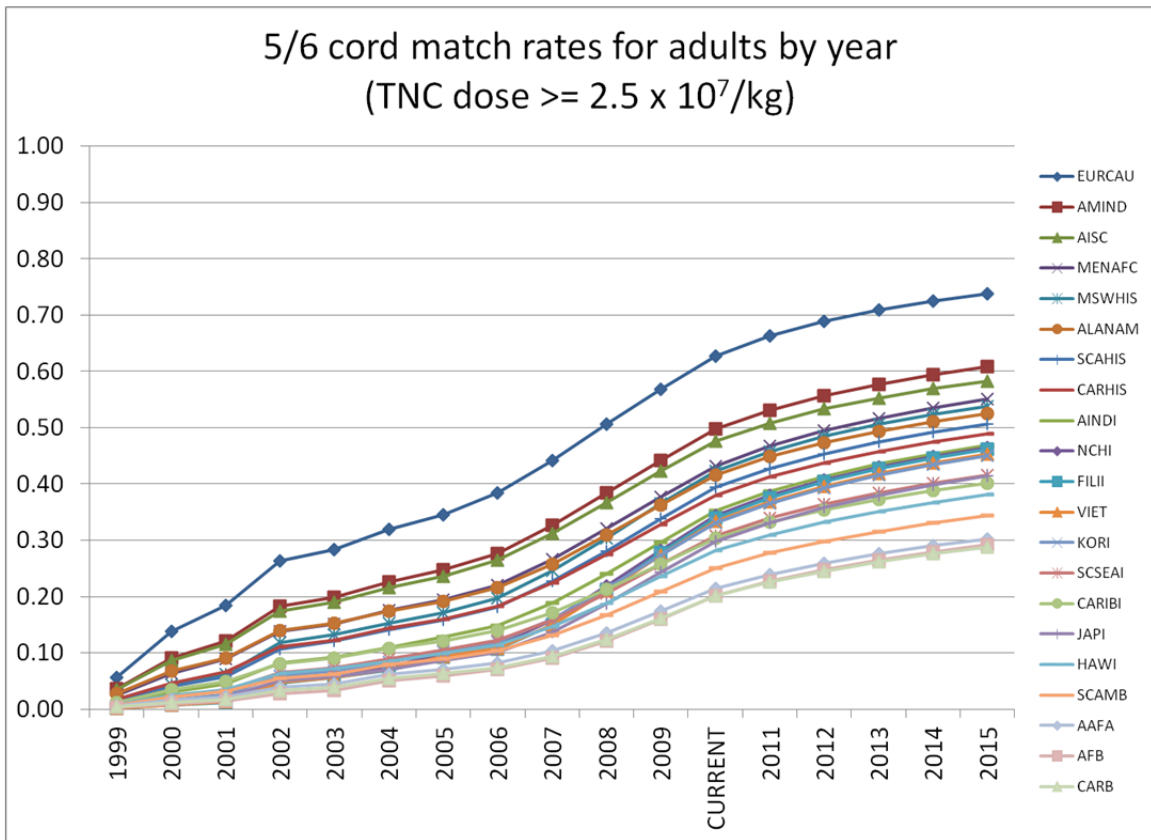
Appendix: Statistical Charts and Graphs

Transplants by Cell Source Pediatric Recipients (younger than 18 years)



Source: National Marrow Donor Program/Be The Match FY 2013





Percent Market Saturation, 0-19 years (Actual / Calculated Demand)

