



The National Marrow Donor Program (NMDP) appreciates the opportunity to respond to the questions presented to Dr. Jeffrey Chell by the Committee. Please do not hesitate to contact us if you would like further clarification or have additional questions.

- 1) You mentioned in your testimony some of the roles that the National Marrow Donor Program plays in addition to running the national registry. Can you elaborate a bit on all the work that Be The Match – NMDP does?*

Operating the National Registry (Be The Match) consists of recruiting potential donors, making them available to searching patients through their transplant physician, and managing the donors safely through the donation process followed by delivering the collected stem cells or cord blood unit to the patient. Although operating the National Registry is the principal role of the NMDP, there are a number of other programs the NMDP has initiated to reduce barriers to transplantation. The National Registry is the Single Point of Access authorized by the Congress.

Assistance to Patients and Families

We know that some populations have extraordinary barriers to accessing transplantation. Family finances, health insurance, delays in diagnosis, treatment or referral, language and literacy all conspire to make it difficult for patients to complete the transplant journey from diagnosis to survivorship. As a tireless patient advocate, NMDP has developed programs to address each of these issues. Our Foundation raises money to help cover expenses that are not covered by insurance. We advocate on behalf of patients with their insurance companies if they are denied coverage. We provide the evidence to payers regarding the effectiveness of transplant therapy. We provide information in multiple languages and have multilingual patient advocacy staff that can provide information and education to patients and families. NMDP provides information and education to non-transplant physicians to help them understand, by diagnosis, the optimal time to refer their patients to a transplant center for assessment. Much of this assistance is provided through the Office of Patient Advocacy authorized by the Congress.

Emergency Preparedness

In addition to our advocacy work, the NMDP operates a multi-organization and multi-agency program called the Radiation Injury Treatment Network (RITN). RITN is prepared to respond to a nuclear accident or act of terror that may cause radiation exposure and bone marrow suppression or failure syndrome. Our contingency planning is robust and tested on a regular basis. These activities are consistent with the requirements of operating the Bone Marrow and Cord Blood Coordinating Centers, which are authorized by the Congress.

Research

Through our Bioinformatics Department and our research organization, the Center for International Blood and Marrow Transplant Research (CIBMTR), the NMDP leads the world in defining the criteria for the best matching donor so that we can continually provide the best source of cells and the best outcomes for our patients. We also conduct research on improving the outcomes of transplant by reducing the complications. This work relates to the operation of the Stem Cell Therapeutic Outcomes Database (SCTOD), which is also authorized by the statute.

- 2) *Can you elaborate on the way that Be The Match – NMDP coordinates internationally and the differences that makes for the possibility of a patient finding a match?*

NMDP is the largest registry in the world with more than 12.5 million adult donors and 209,000 units of cord blood. But there are another 13 million donors and 400,000 cord blood units listed with the other 65 registries and cord blood banks worldwide. Because matching is so critical to a good patient outcome, having access to the inventory of all of the registries and cord blood banks around the world increase the likelihood that we will find a match for a United States patient. And in fact this is the case, with 25% of the donors or cord blood units that best match U.S. patients have come from donors or cord blood units that are found outside the United States.

Through secure electronic connectivity, NMDP can search our Registry in a few minutes and the rest of the world's registries within one business day. If the best donor is an international donor, we can facilitate the collection of that donor's cells or cord blood much like we do for domestic donors and cord bloods so we can meet the needs of the patient on a timely basis.

- 3) *What about research – how has data collection through the registry and other activities led to improved patient outcomes?*

NMDP, through its research affiliate, the Center for International Blood and Marrow Transplant Research (CIBMTR), contract with the federal government to operate the Stem Cell Transplantation Outcomes Database, which collects research quality outcomes data for virtually every allogeneic transplant in the United States. It makes that database available to researchers and support that research with research consultation and design services, biostatistical expertise and other support. But research supported by NMDP is not limited to just the database and CIBMTR has supported more than 900 peer review publications since our inception in the following areas:

Observational Research

Through the SCTOD contract and other means, CIBMTR has compiled the results of nearly all transplants performed in the United States and about 50% of the transplants performed abroad. We provide access to researchers to this database so they can retrospectively review the outcomes of transplant and query the database to determine if changes in treatment approaches have an impact on outcomes. With this large database of over 300,000 transplants, we can learn more effective therapies for even the rarest of diseases.

Clinical Trials

With our research colleagues at transplant centers, we conduct prospective clinical trials that are designed to answer a critical question in improving outcomes of transplant. We work with multiple transplant centers at the same time in each trial so that we can accrue patients to a trial more rapidly. This gives us the answer to the research question earlier so we can communicate the results to the transplant and patient community to allow more rapid dissemination and acceptance of the new approach to improve outcomes.

Health Services Research

Our Health Services Research Department focuses on issues of access to transplant by identifying barriers to access and studying ways to remove them effectively and efficiently. Health Services Research also enables the NMDP to ensure that diverse populations can benefit from our research findings.

Immunobiology Research

This area of research encompasses the science of matching donor to recipient. We have learned that there are multiple genetic and non-genetic factors that impact the identification of the best donor or cord blood for a patient. This department identifies those factors and incorporates them into our searching algorithm and communicate them to the transplant community.

- 4) *You mentioned that current pediatric research focuses not only on malignancies, but also on non-cancer diseases that can still be fatal if untreated, like sickle cell disease. How does Be The Match – NMDP help children with both cancer and non-cancerous diseases?*

Historically, transplant has been used to treat malignant disease. However, there are a number of diseases that are non-malignant in nature that could benefit from a transplant. The first two are Sickle Cell Disease and Thalassemia. Both are diseases of red blood cells which make them less effective at carrying oxygen to our tissues and can cause chronic illnesses and early death. A transplant replaces the dysfunctional red blood cell production system in the bone marrow with that of a healthy normal donor production system thereby curing the disease. Both Sickle Cell Disease and Thalassemia disproportionately impact African Americans and Asians in the United States and cause significant public health issues. These diseases

can be cured early in life and allow these children a healthy life and a brighter future.

There are other non-malignant diseases of childhood generally called Glycogen Storage Diseases. In these diseases children are missing a vital enzyme to aid in eliminating toxins that can build up in the bloodstream. By choosing a donor that is genetically matched and capable of producing the enzyme, we can restore the function of this enzyme in the child's system and restore its natural function. Without this treatment, these children do not develop normally and succumb to their disease early in life.

There is also exciting research using cord blood to potentially treat Autism, brain injuries and autoimmune diseases. It is too early to tell if these treatments will be effective but the future of these treatments is exciting and many, many more patients may benefit from cellular therapy.

5) As you mentioned, the number of transplant for racial and ethnic minority patients has increased substantially from the year 2000 to today. What efforts is the Be The Match – NMDP making to continue to expand the diversity of the registry to ensure that minority patients can find matches?

NMDP actively recruits potential donors every year to expand the size of the registry and replace those who no longer qualify as potential donors. Annually, NMDP's direct efforts add approximately 400,000 new registry members and it works to ensure that half of those recruits are from racial and ethnic minorities. In addition, other organizations including the C. W. Bill Young program at the Department of Naval Research add another 250,000 – 300,000 new members, many of whom represent minority populations. NMDP also works with member cord blood banks to support targeted recruitment of cord blood units in minority populations by providing marketing and education materials and grants to support these efforts. We also partner with minority institutions like Historically Black Colleges and Universities, local churches, tech schools. In addition, we identify leaders in the local community, as well as community members that have donated or received stem cells to share their story.