



May 06, 2014

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Office of Naval Research  
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Arlington, VA 22203-1995

Subject: Final Report of the National Marrow Donor Program®  
Reference: Grant #N00014-11-1-0339 between the Office of Naval Research and the National Marrow Donor Program

Dear LCDR Steele:

In accordance with the requirements of the Referenced Cooperative Agreement, the enclosed subject document is provided as the Final Report for each statement of work task item of the Grant for the period of January 01, 2011 through December 31, 2012.

With this submittal of the Final Report, the National Marrow Donor Program has satisfied the all reporting requirements of the above referenced Grant.

Should you have any questions as to the scientific content of the tasks and the performance activity of this progress report, you may contact our Chief Medical Officer – Dennis Confer, MD directly at 612-362-3425.

Please direct any questions pertaining to the Grant to my attention (612-362-3403 or at [cabler@nmdp.org](mailto:cabler@nmdp.org)).

Sincerely,

Carla Abler-Erickson, M.A.  
Contracts Manager

Enclosure: One (1) copy of subject document

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| <b>14. ABSTRACT</b><br><u>1. Contingency Preparedness:</u> Collect information from transplant centers, build awareness of the Transplant Center Contingency Planning Committee and educate the transplant community about the critical importance of establishing a nationwide contingency response plan.<br><br><u>2. Rapid Identification of Matched Donors :</u> Increase operational efficiencies that accelerate the search process and increase patient access are key to preparedness in a contingency event.<br><br><u>3. Immunogenetic Studies:</u> Increase understanding of the immunologic factors important in HSC transplantation.<br><br><u>4. Clinical Research in Transplantation:</u> Create a platform that facilitates multicenter collaboration and data management. |                         |                                |                                                     |                                                              |                                                                                       |  |
| <b>15. SUBJECT TERMS</b><br>Research in HLA Typing, Hematopoietic Stem Cell Transplantation and Clinical Studies to Improve Outcomes                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                       |                         |                                |                                                     |                                                              |                                                                                       |  |
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| <b>a. REPORT</b><br>U                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                      | <b>b. ABSTRACT</b><br>U | <b>c. THIS PAGE</b><br>U       |                                                     |                                                              | <b>19b. TELEPHONE NUMBER (Include area code)</b><br>612.362.3425                      |  |

**National Marrow Donor Program® N00014-11-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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**January 1, 2011 – December 31, 2012**

| <b>TABLE OF CONTENTS</b> |                                                                           |             |
|--------------------------|---------------------------------------------------------------------------|-------------|
| <b>TASK</b>              | <b>DESCRIPTION</b>                                                        | <b>PAGE</b> |
|                          | Acronym List                                                              | 2           |
|                          | Executive Summary                                                         | 7           |
| <b>IIA</b>               | Contingency Preparedness                                                  | 14          |
| IIA.1.1                  | Secure Interest of Transplant Physicians                                  | 14          |
| IIA.1.2                  | GCSF in Radiation Exposure                                                | 16          |
| IIA.1.3                  | Patient Assessment Guidelines                                             | 20          |
| IIA.1.4                  | National Data Collection Model                                            | 22          |
| IIA.2.1                  | Contingency Response Network                                              | 23          |
| IIA.2.2                  | Develop and Test Standard Operating Procedures                            | 35          |
| IIA.3.1                  | I.S. Business Continuity Planning / Disaster Recovery                     | 36          |
| IIA.3.2                  | Operational Continuity Planning                                           | 36          |
| <b>IIB</b>               | Rapid Identification of Matched Donors                                    | 39          |
| IIB.1.1                  | Increase Registry Diversity                                               | 40          |
| IIB.1.2                  | Evaluate HLA-DRB1 High Resolution Typing                                  | 43          |
| IIB.1.3                  | Evaluate HLA-C Typing of Donors                                           | 43          |
| IIB.1.4                  | Evaluate Buccal Swabs                                                     | 43          |
| IIB.1.5                  | Enhancing HLA Data for Selected Donors                                    | 44          |
| IIB.1.6                  | Maintain a Quality Control Program                                        | 44          |
| IIB.2.1                  | Collection of Primary Data                                                | 45          |
| IIB.2.2                  | Validation of Logic of Primary Data                                       | 45          |
| IIB.2.3                  | Reinterpretation of Primary Data                                          | 45          |
| IIB.2.4                  | Genotype Lists & Matching Algorithm                                       | 45          |
| IIB.3.1                  | Phase I of EM Haplotype Logic                                             | 49          |
| IIB.3.2                  | Enhancement of EM Algorithm                                               | 51          |
| IIB.3.3                  | Optimal Registry Size Analysis                                            | 52          |
| IIB.3.4                  | Target Under-represented Phenotypes                                       | 52          |
| IIB.4.1                  | Expand Network Communications                                             | 54          |
| IIB.4.2                  | Central Contingency Management                                            | 54          |
| IIB.4.3                  | Conduct a transplant center benchmarking analysis                         | 60          |
| IIB.4.4                  | Expand capabilities of collection center and apheresis center network     | 60          |
| <b>IIC</b>               | Immunogenetic Studies                                                     | 61          |
| IIC.1.1                  | Donor Recipient Pair Project                                              | 61          |
| IIC.2.1                  | Analysis of non-HLA Loci                                                  | 63          |
| IIC.2.2                  | Related Pairs Research Repository                                         | 67          |
| IIC.2.3                  | CIBMTR Integration                                                        | 67          |
| <b>IID</b>               | Clinical Research in Transplantation                                      | 68          |
| IID.1.1                  | Observational Research, Clinical Trials and NIH Transplant Center         | 68          |
| IID.1.2                  | Research with NMDP Donors                                                 | 75          |
| IID.1.3                  | Expand Immunobiology Research                                             | 75          |
| Attachment A             | References                                                                | 78          |
| Attachment B             | Listing of Published Manuscripts and Abstracts associated with this Grant | 82          |

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**ACRONYM LIST**

|         |                                                               |
|---------|---------------------------------------------------------------|
| AABB    | AABB, formerly the American Association of Blood Banks        |
| AAFA    | African American (NMDP race code)                             |
| ABD     | Antigen Binding Domain                                        |
| AC      | Apheresis Center                                              |
| AFA     | African American                                              |
| AFB     | African                                                       |
| AGNIS®  | A Growable Network Information System                         |
| AIM     | Ancestry Informative Markers                                  |
| AINDI   | South Asian                                                   |
| AISC    | American Indian South or Central                              |
| ALANAM  | Alaska Native or Aleut                                        |
| ALDH    | Aldehyde Dehydrogenase                                        |
| ALDHbr  | Aldehyde Dehydrogenase bright                                 |
| ALL     | Acute Lymphoblastic Leukemia                                  |
| AMIND   | North American Indian                                         |
| AML     | Acute Myelogenous Leukemia                                    |
| API     | Asian Pacific Islander                                        |
| ARC GIS | ArcGIS is a brand name: GIS = Geographical Information System |
| ASBMT   | American Society for Blood and Marrow Transplantation         |
| ASH     | American Society of Hematology                                |
| ASHG    | American Society of Human Genetics                            |
| ASHI    | American Society for Histocompatibility and Immunogenetics    |
| ASPR    | Assistant Secretary for Preparedness and Response             |
| B2B     | Business to Business                                          |
| BARDA   | Biomedical Advanced Research and Development Authority        |
| BBMT    | Biology of Blood and Marrow Transplant                        |
| B-LCLs  | B-Lymphocytic Cell Lines                                      |
| BMDW    | Bone Marrow Donors Worldwide                                  |
| BMT     | Bone Marrow Transplant/Transplantation                        |
| BMT CTN | Blood and Marrow Transplant - Clinical Trials Network         |
| BODI    | Business Objects Data Integrator                              |
| BRIDG   | Biomedical Research Integrated Domain Group                   |
| caDSR   | Cancer Data Standards Repository                              |
| CARB    | Black Caribbean                                               |
| CARHIS  | Caribbean Hispanic                                            |
| CARIBI  | Caribbean Indian                                              |
| CAU     | Caucasian                                                     |
| CB      | Cord Blood                                                    |
| CBB     | Cord Blood Bank                                               |
| CBT     | Cord Blood Transplantation                                    |
| CBU     | Cord Blood Unit                                               |

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**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

|         |                                                                  |
|---------|------------------------------------------------------------------|
| CC      | Collection Center                                                |
| CEM     | Certified Emergency Manager                                      |
| CEO     | Chief Executive Officer                                          |
| CFO     | Chief Financial Officer                                          |
| CFU     | Colony Forming Unit                                              |
| CIBMTR® | Center for International Blood & Marrow Transplant Research      |
| CIO     | Chief Information Officer                                        |
| CML     | Chronic Myelogenous Leukemia                                     |
| CMO     | Chief Medical Officer                                            |
| CMS     | Center for Medicare and Medicaid Services                        |
| CRIS    | Computerized Repository Inventory System                         |
| CSF     | Colony Stimulating Factors                                       |
| CT      | Confirmatory Testing                                             |
| CTA     | Clinical Trial Application                                       |
| CTAC    | Clinical Trials Advisory Committee                               |
| DAIT    | Division of Allergy, Immunology, and Transplantation             |
| DC      | Donor Center                                                     |
| DHHS    | Department of Health and Human Services                          |
| DKMS    | Deutsche Knochenmarkspenderdatei                                 |
| DNA     | Deoxyribonucleic Acid                                            |
| DoD     | Department of Defense                                            |
| D/R     | Donor/Recipient                                                  |
| DR      | Disaster Recovery                                                |
| DQ      | Data Quality                                                     |
| DNA     | Deoxyribonucleic Acid                                            |
| DR      | Disaster Recovery                                                |
| D/R     | Donor/Recipient                                                  |
| EBMT    | European Group for Blood and Marrow Transplantation              |
| EC      | Ethics Committee                                                 |
| EFI     | European Federation for Immunogenetics                           |
| ELISA   | Enzyme-linked Immunosorbant Assay                                |
| EM      | Expectation Maximization                                         |
| EMDIS   | European Marrow Donor Information System                         |
| ESRI    | Environmental Systems Research Institute                         |
| FACS    | Fluorescent Activated Cell Sorting                               |
| FILII   | Filipino                                                         |
| FLOCK   | Flow Cytometry Analysis Component                                |
| FY      | Fiscal Year                                                      |
| GETS    | Government Emergency Telecommunications Service                  |
| GCSF    | Granulocyte-Colony Stimulating Factor (also known as filgrastim) |
| GIS     | Geographic Information System                                    |
| GM-CSF  | Granulocyte Macrophage Colony Stimulating Factor                 |

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**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

|           |                                                              |
|-----------|--------------------------------------------------------------|
| GVHD      | Graft vs. Host Disease                                       |
| GWAS      | Genome Wide Association Studies                              |
| Gy        | Gray-measure of dose of irradiation                          |
| HAWI      | Hawaiian or other Pacific Islander Unspecified               |
| HC        | Hematopoietic Cell                                           |
| HCT       | Hematopoietic Cell Transplantation                           |
| HHQ       | Health History Questionnaire                                 |
| HHS       | Health and Human Services                                    |
| HIS       | Hispanic                                                     |
| HIV       | Human Immunodeficiency Virus                                 |
| HLA       | Human Leukocyte Antigen                                      |
| HML       | Histoimmunogenetics Mark-up Language                         |
| HR        | High Resolution                                              |
| HRSA      | Health Resources and Services Administration                 |
| HSC       | Hematopoietic Stem Cell                                      |
| HSCT      | Hematopoietic Stem Cell Transplant                           |
| IBMTR     | International Bone Marrow Transplant Registry                |
| IBWC      | Immunobiology Working Committee                              |
| IDM       | Infectious Disease Markers                                   |
| Ig        | Immunoglobulin                                               |
| IHIWS     | International Histocompatibility Work Shop                   |
| IHWG      | International Histocompatibility Working Group               |
| IIDB      | Immunobiology Integration Database                           |
| IIMMS     | International Immunomics Society                             |
| IMGT      | ImMunoGeneTics                                               |
| ImmPort   | Immunology Database and Analysis Portal                      |
| IPD       | Immuno Polymorphism Database                                 |
| IPR       | Immunobiology Project Results                                |
| IRB       | Institutional Review Board                                   |
| IS        | Information Services                                         |
| IT        | Information Technology                                       |
| JAPI      | Japanese                                                     |
| KIR       | Killer Immunoglobulin-like Receptor                          |
| KIR-DS    | Killer Immunoglobulin-like Receptor Donor Selection          |
| KORI      | Korean                                                       |
| LD        | Linkage Disequilibrium                                       |
| LTA       | Lymphotoxin Alpha                                            |
| MALDI-TOF | Matrix-Assisted Laser Desorption/Ionization – Time Of Flight |
| MCW       | Medical College of Wisconsin                                 |
| MD        | Medical Doctor                                               |
| MDACC     | MD Anderson Cancer Center                                    |
| MDS       | Myelodysplastic Syndrome                                     |

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

|                  |                                                                          |
|------------------|--------------------------------------------------------------------------|
| MENAFC           | MidEast/North Coast of Africa                                            |
| MHC              | Major Histocompatibility Complex                                         |
| MICA             | MHC Class I-Like Molecule, Chain A                                       |
| MICB             | MHC Class I-Like Molecule, Chain B                                       |
| mHA <sub>g</sub> | Minor Histocompatibility Antigen                                         |
| MOU              | Memorandum of Understanding                                              |
| MRD              | Minimal Residual Disease                                                 |
| MSKCC            | Memorial Sloan-Kettering Cancer Center                                   |
| MSWHIS           | Mexican or Chicano                                                       |
| NAM              | Native American                                                          |
| NAMER            | North American                                                           |
| NCBI             | National Center for Biotechnology Information                            |
| NCBM             | National Conference of Black Mayors                                      |
| NCHI             | Chinese                                                                  |
| NCI              | National Cancer Institute                                                |
| NECEP            | New England Center for Emergency Preparedness                            |
| NHLBI            | National Heart Lung and Blood Institute                                  |
| NIAID            | National Institute of Allergy and Infectious Diseases                    |
| NIH              | National Institutes of Health                                            |
| NIMA             | Non-inherited maternal antigen                                           |
| NK               | Natural Killer                                                           |
| NL               | Netherlands                                                              |
| NLM              | National Library of Medicine                                             |
| NMDP®            | National Marrow Donor Program                                            |
| NST              | Non-myeloablative Allogeneic Stem Cell Transplantation                   |
| OCP              | Operational Continuity Plan                                              |
| ONR              | Office of Naval Research                                                 |
| PA               | Physician's Assistant                                                    |
| PBSC             | Peripheral Blood Stem Cell                                               |
| PCR              | Polymerase Chain Reaction                                                |
| PI               | Principal Investigator                                                   |
| QC               | Quality control                                                          |
| RadCCore         | Radiation Countermeasures Center of Research Excellence                  |
| RCI              | Resource for Clinical Investigations                                     |
| RCI BMT          | Resource for Clinical Investigations in Blood and Marrow Transplantation |
| RD Safe          | Related Donor Safety                                                     |
| REAC/TS          | Radiation Emergency Assistance Center/Training Site                      |
| REMM             | Radiation Event Medical Management                                       |
| RFP              | Request for Proposal                                                     |
| RFQ              | Request for Quotation                                                    |
| RITN             | Radiation Injury Treatment Network                                       |
| RT-PCR           | Reverse Transcriptase-Polymerase Chain Reaction                          |

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**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

|            |                                                                                            |
|------------|--------------------------------------------------------------------------------------------|
| SAA        | Severe Aplastic Anemia                                                                     |
| SBT        | Sequence Based Typing                                                                      |
| SCAHIS     | South/Central American Hispanic                                                            |
| SCAMB      | Black South or Central America                                                             |
| SCSEAI     | Southeast Asian                                                                            |
| SCT        | Stem Cell Transplantation                                                                  |
| SCTOD      | Stem Cell Therapeutics Outcome Database                                                    |
| SG         | Sample Group                                                                               |
| SNP        | Single Nucleotide Polymorphism                                                             |
| SOP        | Standard Operating Procedure                                                               |
| SRG        | Survey Research Group                                                                      |
| SSO        | Sequence Specific Oligonucleotides                                                         |
| SSP        | Sequence Specific Primers                                                                  |
| SSOP       | Sequence Specific Oligonucleotide Probes                                                   |
| STAR®      | Search, Tracking and Registry                                                              |
| TBI        | Total Body Irradiation                                                                     |
| TC         | Transplant Center                                                                          |
| TNC        | Total Nucleated Cell                                                                       |
| UCB        | Umbilical Cord Blood                                                                       |
| UCBT       | Umbilical Cord Blood Transplant                                                            |
| UI         | User Interface                                                                             |
| UML        | Unified Modeling Language                                                                  |
| URD        | Unrelated Donor                                                                            |
| US         | United States                                                                              |
| USB        | Universal Serial Bus                                                                       |
| VP         | Vice President                                                                             |
| VIET       | Vietnamese                                                                                 |
| WebEOC®    | Web-based Emergency Operations Center                                                      |
| WGA        | Whole Genome Amplification                                                                 |
| WHO        | World Health Organization                                                                  |
| WHO-REMPAN | World Health Organization, Radiation Emergency Medical Preparedness and Assistance Network |
| WMDA       | World Marrow Donor Association                                                             |
| WU         | Work-up                                                                                    |
| XML        | Extensible Markup Language                                                                 |
| ZKRD       | Zentrales Knochenmarkspender – Register für die Bundesrepublik Deutschland                 |

**National Marrow Donor Program® N00014-11-1-0339**  
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## **Executive Summary**

In 1986, Congress appropriated funds to begin development of the National Bone Marrow Donor Registry. Today, 26 years later, the National Marrow Donor Program (NMDP), as the contractor for the Registry, has built a racially diverse donor registry of more than 10.5 million donors, facilitated more than 50,000 hematopoietic stem cell transplants, developed comprehensive research programs to improve post-transplant outcomes, and established a network of transplant centers (TCs) capable of treating casualties resulting from military or terrorist actions, as well as patients suffering from leukemia, aplastic anemia, and other life-threatening diseases.

### **Contingency Preparedness Planning**

As a result of grant support, the Radiation Injury Treatment Network® (RITN) grew in size and capability, and the visibility of RITN within the public health preparedness community was increased. New partnerships were solidified that will allow for development of future preparedness activities with regional, national, and international entities. RITN broke new ground in response to the Fukushima nuclear power plant emergency by providing situation reports to the network as well as our partners. These situation reports were a concise summary of many reports from organizations, governments, and open source news agencies, allowing readers to quickly review the status. RITN also coordinated multiple research, development and training opportunities for healthcare workers and the preparedness community.

Continued development of the NMDP's organizational resiliency program was refined and tested to show that our capabilities have improved, confirming that we are better prepared for a catastrophic operational interruption.

### **Rapid Identification of Matched Donors**

Published research data have clearly defined the relationship between HLA matching and optimal patient outcomes following unrelated adult donor transplantation. Continually working to increase the genetic diversity of the Registry helps to ensure that more patients will be able to locate a suitably matched stem cell product for a transplant.

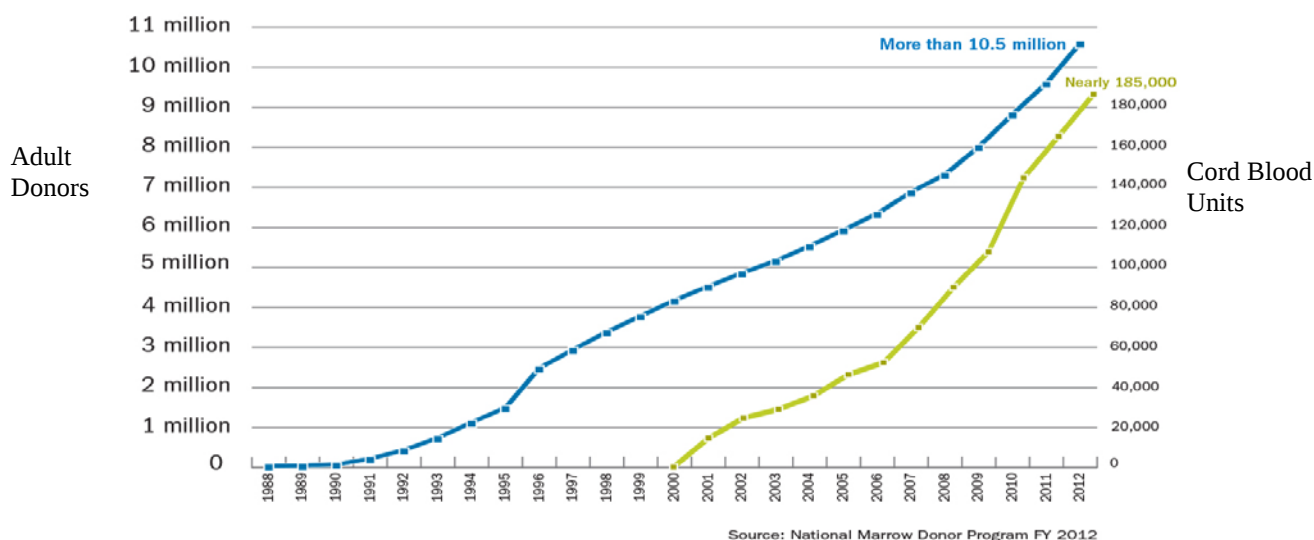
During NMDP FY11, NMDP donor centers (including DoD) and recruitment groups recruited 259,612 minority race and 249,104 Caucasian donors, for a total of 508,716 U.S. donors added to the Registry. Navy funding contributed to the addition of 143,280 of this culturally diverse group of new donors, with 46,834 of these donors being minorities. All donors were typed for a minimum of HLA-A, B, and DRB1.

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**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Advances in laboratory methods and technology continue to have a positive impact on lab performance and pricing. As of December, 2012:

- 97% of new donors received higher than intermediate HLA-A, B and DRB1 typing
- 52% of new donors received additional locus testing (HLA-C, DQB1 and DPB1)
- Blind quality control testing error rate was 0.08%, exceeding the project requirement of  $\leq 2.0\%$ .
- On-time testing completion rate was 99%, meeting the project requirement of a minimum of 90% of typing results reported within 14 days of shipment of samples.
- The cost of HLA typing continues to decrease as technology improves; in December 2012, the average price per sample was approximately \$40.00 compared to \$134.75 (\$181.17 in 2012 dollars) in 1997, which represents a decrease of over 78%.

**Be the Match Registry Growth: Adult Donors and Cord Blood Units**



During this grant period, the NMDP completed the *7/8 High Resolution Donor Match Rate Study*. A previous Navy funded research project (FY10) estimated the 8/8 high resolution donor match rate for the four largest NMDP patient populations: Caucasian (CAU), African American (AFA), Hispanic (HIS), and Asian-Pacific Islander (API). This study used a simulated patient cohort of “pseudo-patients”. As a second phase, a research project was developed and completed this period to estimate the 7/8 HLA high resolution donor match rate, given this is a typically acceptable match level for transplantation of an unrelated donor. The project also re-evaluated the 8/8 high resolution match rate which was previously determined using a 2009 donor registry snapshot. A total of 5130 donors and 6386 loci were typed during this project period. 9/10 and 10/10 match rates, which include HLA-DQB1, were also determined for all four race groups. Results (nearly complete) show disparity in 7/8 match levels between CAU (~98%) and non-CAU patients (~81-87%). The data relooking at the 8/8 match rate also showed improvement

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

from the 2009 dataset match rate of ~2-3% for each group. This represents new donor recruitment gains over the 3 year maturation of the NMDP registry.

During the grant period, NMDP completed development and implemented an enhanced version of the HapLogic matching algorithm that provided increased match precision, expanded HLA locus predictions and improved performance including:

- 3 locus matching → 5 locus matching
- x of 6 → x of 8, x of 10 predictions
- 5 broad race groups → 5 broad and 18 detailed race groups
- Ensuring visibility of NMDP's best matched donors and cords
- More precision for mismatch searches
- Better aligned with clinical practice
- Continued reduction in search run times
- Achieved median search run time of 35 seconds

### Immunogenetic Research

The high resolution HLA typing of paired donor and recipient samples continued to provide substantive data to increase the understanding of the impact of HLA matching on patient outcome. The project data were also used to assess genetic diversity within the NMDP transplant population and Registry, and fed into the HapLogic matching algorithm. Testing was completed on an additional 110 donor/recipient and 295 CBU/recipient pairs during the project period, bringing the total enrolled to over 15,000. Typing at the HLA-DPB1 locus was added back to facilitate future studies of HLA-DPB1 matching. Presence/absence Killer Immunoglobulin-like Receptor (KIR) genotyping on 2DL1-5, 2DS1-5, 3DL1-3 and 3DS1 has continued. To date over 2400 pairs and 1180 additional donors have been typed for presence/absence of 14 KIR loci (2DL1-5, 2DS1-5, 3DL1-3 and 3DS1).

In order to continuously upgrade the Donor/Recipient Pairs Project, and include as many CBU/recipient pairs as possible, we have begun to include pairs with Whole Genome Amplification (WGA) DNA from the Repository. Samples of recipients and CBUs with limited blood or DNA samples in the research repository were selected and sent for WGA. WGA of these samples allowed for the inclusion of 295 CBU/recipient pairs during the grant period.

The high-resolution HLA data generated through the project are routinely incorporated into all outcomes analyses performed by the NMDP research program, the Center for International Blood and Marrow Transplant Research (CIBMTR), to provide the best HLA typing and matching information possible. The project has developed the largest, fully validated pool of unrelated stem cell transplant donor-recipient HLA data in the world and is an unparalleled resource for transplant research. The data generated through the project have had a major impact on the evolution of the NMDP HLA matching requirements.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Current HLA matching guidelines for unrelated Hematopoietic Cell Transplantation (HCT) recommend avoidance of mismatches only within the antigen recognition site, i.e., exons 2 and 3 for HLA class I and exon 2 for HLA class II. This recommendation is based on the hypothesis that amino acid differences outside the antigen recognition site are not immunogenic. The Antigen Recognition Site Allo-reactivity Assessment Project will give insight into the allowable tolerance of matching needed outside of this binding region. Preliminary results on DRB1\*14:01 vs. \*14:54 suggest that the mismatch may be less alloreactive than mismatches within the antigen binding domain.

## Clinical Research in Transplantation

### **Prospective Research Activity**

During this grant, the Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT), a division within CIBMTR, developed its infrastructure further and made progress on a number of studies. The goal of this program is to provide an avenue for investigators to obtain statistical, study and data management support for prospective trials focusing on addressing various transplant issues. The following key elements were accomplished:

- The Clinical Trials Advisory Committee (CTAC), whose mission is to provide scientific review and recommendations on clinical trial proposals, met a total of three times during this period.
- RCI BMT managed the Adult Double Cord Blood trial in patients with hematologic malignancies. During this grant period, accrual was met with a total of 56 patients enrolling. Staff continued to coordinate study activities, manage data and perform on site monitoring. A Tandem 2013 abstract was developed, submitted and accepted for oral presentation. A manuscript is submitted and under review.
- The Lenalidomide after allogeneic HCT for Myeloma trial met its accrual goal with 30 patients. Data management activities continued and a Tandem 2013 abstract was accepted for oral presentation.
- Staff supported the continued efforts related to the BMT CTN PBSC vs Marrow Phase III trial. This included managing the donor follow up component of the study and site monitoring at the donor centers. During this grant the Survey Research Group (SRG) acquired the five-year time point of the recipient quality of life and fully integrated this effort into their activities.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- The Long-Term Donor Follow-up protocol continued to accrue donors both from the previous donated group and prospectively as donors participated in the standard work-up process. At the end of this grant period close to 10,000 donors were enrolled.
- Study accrual and data submission in support of the CMS MDS study. Transplant Centers with a recipient enrolled on this study are required to submit the comprehensive research forms. A total of 82 recipient comprehensive report forms (CRF) were completed for this study during this period.
- RCI BMT staff worked with CIT staff to complete operational requirements for a comprehensive system for management of activities and studies within the SRG and clinical trial management system (CTMS) to coordinate operational and administrative activities within RCI BMT.

Support of the Observational Research program included statistical support for managing studies within the Immunobiology, GVHD, and Graft Sources Working Committees. During this grant period, staff performed proposal review, protocol development, data preparation, data analysis, and manuscript preparations. Details regarding the Immunobiology activities can be found in IID1.3 below. The GVHD and Graft Sources Working Committees published 9 manuscripts. During the grant period, staff supported progress on over 20 other studies.

### **Cord Blood Research Activity**

During the project period, the Cord Blood Research sub-Committee met semi-monthly to discuss study priorities and plan analyses for the following:

- The Duke and MD Anderson laboratory staff completed work on validating the assay methodologies but were unable to ensure consistent results generated at both testing sites for the study investigating biomarkers associated with cord blood engraftment. Initial and final statistical analysis of the validation testing results showed poor inter-laboratory reliability for all assays performed. Therefore, testing using a third laboratory was developed with St. Louis Cord Blood Bank (SLCBB) to determine whether the poor reliability results from center-specific or assay-related issues.
- The Duke cord blood bank and St. Louis Cord Blood Bank (SLCBB) created and finalized plans and testing for training and validating the assay methodologies. The data analysis for the results of the training phase met the acceptable threshold for the inter-laboratory reliability coefficient of variation (CV); however, the validation phase data analysis did not. An investigation into the cause of the poor reliability is on-going and does not involve further testing of samples.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- A white paper detailing recommendations/guidelines for the assessment of new assays (potency or other assays) relevant to cord blood banking and/or transplantation was published in Cytotherapy. As a result, the NMDP was contacted by Hemogenix regarding a collaborative validation study for their potency assay (HALO).
- An analysis evaluating the likelihood of finding a non-inherited maternal antigen/allele (NIMA) match for HLA mismatched cord blood unit for transplant when upfront maternal typing is not available was completed. The retrospective analysis compared the frequencies of the NIMA matched and mismatched HLA- A, B antigens or DRB1 alleles found in the Eurocord/NMDP/CIBMTR study to determine any significant differences. Results were incorporated into a manuscript and submitted to and published in BBMT.
- Work began on a study to assess CBU characteristics (viability, TNC, CFU and CD34) pre-freeze and post thaw. Segment evaluation prior to unit release is under consideration as a third evaluation point, but will require an understanding of the release testing performed by the various CBBs. A survey was sent out to Network cord blood banks to collect data on cord blood release testing practices. The study design and proposal for submission to the CIBMTR was completed, submitted and accepted for presentation at the Graft Sources and Manipulation Working Committee at Tandem in 2013.
- Development of the anti-HLA donor specific antibody study of recipients transplanted with cord blood units was initiated. It was determined that the study cohort was too small to proceed with the study during the grant period.
- Work was initiated and completed on an assessment of the impact of donor inherited paternal antigen (IPA) disparity on outcomes after unrelated CBU transplantation for acute lymphoblastic leukemia and acute myelogenous leukemia. Results were detailed in an abstract submitted and accepted for poster presentation at the 2012 International Cord Blood Symposium.
- Two cord blood workshops were developed and conducted at both the 2011 and 2012 NMDP Council Meetings. Each workshop was well attended and received excellent ratings from the attendees.

### **CIBMTR IT Activity**

- During the project period, significant strides were made in improving user experience, delivering new functionality, improving data quality, data capture and data reporting through the CIBMTR IT suite of applications.

### **Immunobiology Research Activity**

To further stimulate completion of immunobiology studies within the CIBMTR, grant funds were used to provide support to investigators that required supplemental funding to cover

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

research sample access costs. One grant was awarded during the grant period. Grant funds also supported Immunobiology Working Committee (IBWC) leadership outreach activities to promote the activities and resources of the committee to the scientific community. The committee maintained a strong performance record with 6 abstracts, 10 publications (submitted or accepted) during the grant period. In addition, 6 new proposals were accepted by the IBWC during the BMT Tandem meetings in February 2012.

**END – EXECUTIVE SUMMARY**

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.A. Contingency Preparedness – Hypothesis 1:**

Recovery of casualties with significant myelosuppression following radiation or chemical exposure will be optimal when care plans are designed and implemented by transplant physicians.

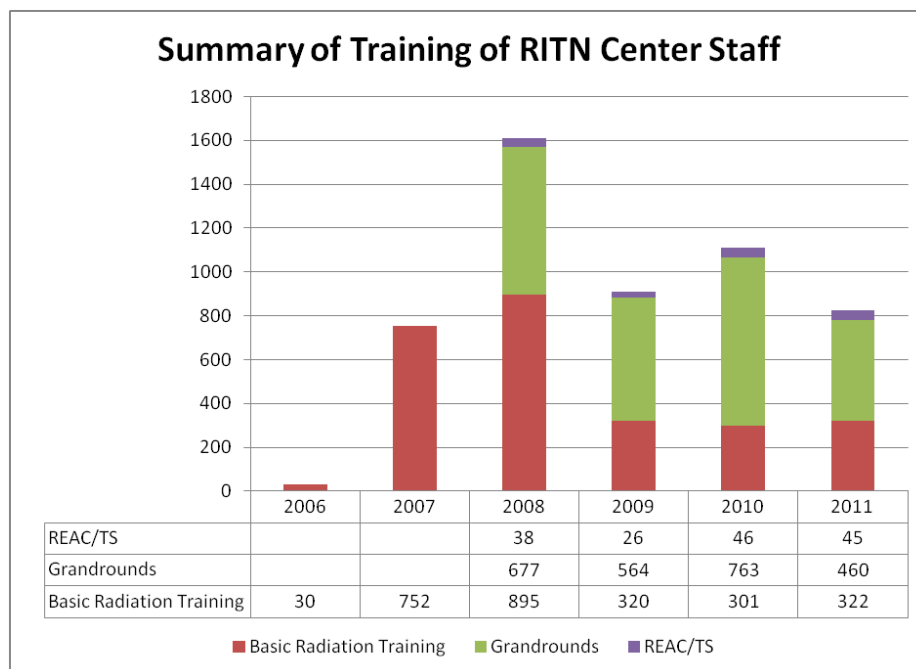
### **Aim A.1.1: Secure Interest of Transplant Physicians**

This Aim was focused on the education of transplant physicians and their staff from Network centers affiliated with the Radiation Injury Treatment Network® (RITN). RITN centers are required to complete educational tasks as part of their membership each year

RITN training efforts began with a short training course on Basic Radiation Training that was introduced in 2006. The course provided an hour of Continuing Medical Education credit to attendees and was quickly embraced by the transplant community. In 2008, a standardized medical grand rounds training course was developed for presentation at medical staff at RITN hospitals. In addition, RITN began to coordinate training of medical staff at the Radiation Emergency Assistance Center and Training Site (REAC/TS).

As depicted in Figure 1 below, 2,620 staff successfully completed the Basic Radiation Training course since 2006: 2,464 completed the Acute Radiation Syndrome Medical Grand Rounds and 155 partners completed the Advanced Radiological Medical Response at the Radiation Emergency Assistance Center and Training Site.

**Figure 1: Table summarizing RITN training from 2006-2011**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Training conducted in Oak Ridge, Tennessee at the Radiation Emergency Assistance Center and Training Site is an intense two day Advanced Medical Training on Radiation Emergency Medicine course. Attendees earn 14 continuing medical education credits for attending this rigorous course which covers a comprehensive set of topics including:

- Basic Health Physics & Radiation Protection: Part I
- A History of Serious Radiological Incidents: The Real Risk
- Health Physics & Contamination Control: Part II
- Radiation Detection, Monitoring & Protection Laboratory Exercise & Quiz
- Diagnosis & Management of the Acute Radiation Syndrome
- Diagnosis & Management of Internal Contamination
- Diagnosis & Management of Acute Local Radiation Injury & Case Review
- Radiation Sources & Radiological Terrorism
- Radiation Emergency Area Protocol Demonstration
- Radiation Emergency Medical Management Drill
- Radiation Dose Estimations – Problem Solving Session

One limitation to the available RITN courses has been the method of delivery. With this in mind, a web based learning management system was procured to allow development and deployment of web based training. The web based system will allow more people to complete the training provided and allow for instantaneous test grading with on demand printing of certificates of completion. To present a consistent image and develop professional training materials, an Instructional Designer was hired to formulate a theme and organize the approach to the web based training materials. The approach that was developed is to have multiple training modules that are all independent, yet when taken as a group, can be used to orient new members of the RITN. Modules that were completed and released through this grant include:

1. Introduction to the Radiation Injury Treatment Network
2. Basic Radiation Training
3. Government Emergency Telecommunications Service Calling Card Usage
4. Satellite Telephone Training for the Radiation Injury Treatment Network

Additional courses that will be completed under a subsequent grant include:

5. Radiation Injury Treatment Network Concept of Operations Training
6. Basic Radiation Training for Non-medical Staff

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 2. Screenshots of the new RITN Training Materials**



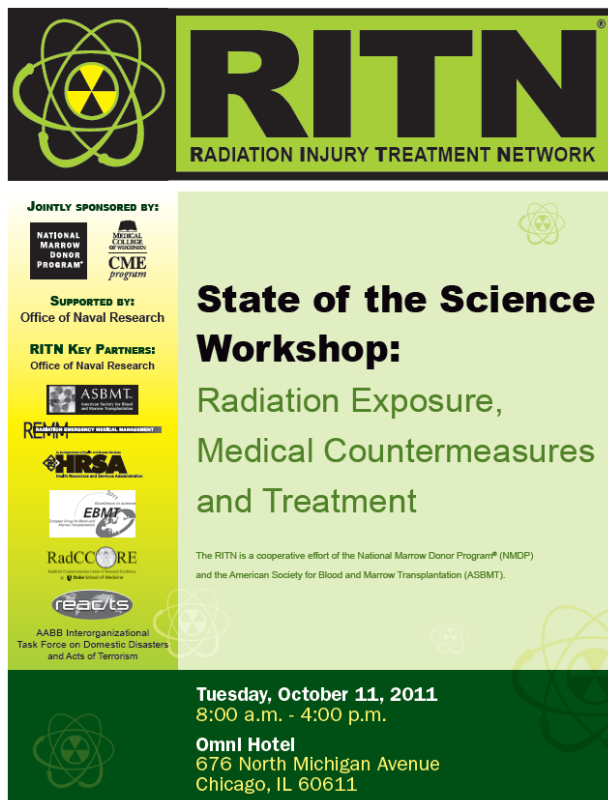
**Aim A.1.2: GCSF in Radiation Exposure**

This Aim focused on non-transplant treatment guidelines and patient assessment related to the use of granulocyte colony stimulating factor (GCSF) for patient treatment as a result of a marrow toxic mass casualty incident such as exposure to ionizing radiation.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**2011 RITN State of the Science Workshop: Radiation Exposure, Medical Countermeasures and Treatment**

In support of the development of non-transplant treatment protocols (Aim A.1.2), transplant treatment protocols (Aim A.1.3), physician education (Aim A.1.1) and the RITN (Aim A.2.1), a conference was held on October 11, 2011 in Chicago, IL. The RITN *State of the Science Workshop: Radiation Exposure, Medical Countermeasures and Treatment* was attended by 125 physicians, medical staff, researchers, public health officials, and emergency planners.



The purpose of the conference was to highlight the following:

*Response to a radiological mass casualty incident which would stress the capacity of the entire U.S. medical community. Through optimization of care and thoughtful planning, some of the resource gaps can be closed and resiliency improved. Presentations provided updates on research in the biology of radiation exposure, biodosimetry, supportive care, immune reconstitution, and medical countermeasures.*

With the following educational objectives:

1. Describe developments in the research of biological effects of exposure to ionizing radiation.
2. Explain currently available and research methods for measuring the dose of radiation absorbed by victims.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

3. Outline organ system and systemic responses to ionizing radiation exposure.
4. Describe the impact of the Japanese Nuclear Power Plant incident on clinical and research infrastructure.
5. Discuss the path to preparedness for a mass casualty incident with injuries due to the exposure to ionizing radiation.

The agenda presented a balance between unpublished updates on current research to operational strategies to respond to the detonation of an improvised nuclear device:

1. “Where We Are and Where We Are Going”-Richard Hatchett (DHHS-BARDA)
2. Biodosimetry:
  - a. High-Throughput Minimally-Invasive Radiation Biodosimetry - Sally Amundson (Columbia)
  - b. Informing Biosignatures with Data from Model Systems - Joseph Lucas (Duke)
  - c. Physical Biodosimetry for Triage After a Large Scale Radiation Event - Harold Swartz (Dartmouth)
3. Supportive Care: Medical Countermeasures for the ARS: Evidence Based Support and the FDA Animal Rule - Tom MacVittie (Univ. Maryland)
4. Hematopoietic and Immune System Reconstitution:
  - d. Combined Radiation Injury - Nelson Chao (Duke)
  - e. Stem Cell-Based Therapies for Acute Radiation Syndrome - Chandan Guha (Albert Einstein College of Medicine)
  - f. Novel Mitigators of the Hematopoietic Syndrome - John Chute (Duke)
  - g. Strategies to Enhance Immune Reconstitution Post Irradiation - Marcel van den Brink (Memorial Sloan Kettering Cancer Center)
5. Organ Toxicity:
  - h. Pulmonary Toxicity - Zeljko Vujaskovic (Duke)
  - i. Gastrointestinal Organ Toxicity - Martin Hauer Jensen (Univ. of Arkansas for Medical Sciences)
6. Radiation Biology:
  - j. Design of Mitochondria-Targeted Radio Protectors/Mitigators - Valerian Kagan (Univ. Pittsburgh)
  - k. The Role of P53 in Acute Radiation Injury and Late Effects from Radiation - David Kirsch (Duke)
7. Guided Discussion: Autologous Cell Collection for Radiological Disaster Responders - David Weinstock (Dana Farber Cancer Institute)

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

8. Global Consensus on Evidence-Based Management of Acute Radiation Syndrome - Nickolas Dainiak (Yale) and Viktor Meineke (Bundeswehr-Germany Military)
9. Keynote: Experience in Japan During the Fukushima Incident - Robert Bazell (NBC News)

Overall feedback from attendees was excellent. Sessions and speakers were highly rated in conference evaluations, scoring 4.67 out of a possible of 5.0. All evaluators reported learning something new during the conference and 85% of the evaluators indicated that they will directly apply information learned in their practice.

### **Post-Conference Review of Logistical Issues During Incident Response**

The day following the conference, a closed meeting was held to review three key concepts related to a radiological incident:

1. Define when a victim crosses the threshold from solely requiring intensive supportive care to requiring HCT.
2. Determine best practices used to expand available care when confronted with mass casualties with marrow toxic injuries.
3. Determine what level of additional review is necessary to gather agreement for each area.

The outcomes of these discussions were incorporated in an update to the RITN Acute Radiation Syndrome Treatment Guidelines described in Aim 1.3 below.

### **Aim A.1.3: Patient Assessment Guidelines**

This Aim focused on the development of transplant treatment guidelines and the associated support systems, including the refinement of guidelines for patient assessment and the operational and educational aspects of rapid product selection and transplant as necessary in a marrow toxic mass casualty event such as radiation exposure.

Activity under this aim was incorporated into the RITN educational conference for medical staff, researchers, and public health officials entitled *State of the Science Workshop: Radiation Exposure, Medical Countermeasures and Treatment* that was described in detail under Aim A.1.2 section.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Aim A.1.3: Patient Assessment Guidelines and System Enhancements**

**Review of Logistical Issues During Incident Response**

A one day meeting was held in conjunction with the State of the Science Workshop to discuss updates to the RITN Acute Radiation Syndrome Treatment Guidelines. The following key results were solidified and used to update the guidelines:

- 1. Which products (bone marrow, peripheral blood stem cells, and umbilical cord blood) would be preferred for a radiological casualty?**
  - a. Bone marrow will likely be the preferred option as GCSF will be in limited supply as most available stock is redirected to the Strategic National Stockpile (SNS). GCSF in the SNS is currently limited to 5,000 doses although there is additional likely to be available through vendor-managed inventory.*
  - b. Cord blood units will be an alternative.*
  - c. This could raise issues for patient treatment that requires growth factors.*
- 2. What are the appropriate preparative regimens for transplantation?**
  - a. The existing RITN treatment guidelines, based on a standard regimen for patients with aplitic anemia, remains a reasonable option*
  - b. Guidelines for growth factors and other supportive care after transplantation are not included*
- 3. Can a center handle 30 HCT in a 2 week period?**
  - a. Assuming that only ~10% of the 300 patients at each center require transplant and the rest only require observation and supportive care.*
  - b. Need to determine what the limited resources would be for 30 HCTs.*
  - c. S3 or G3 will not be a HCT candidate until condition improves and are stabilized (S3 refers to significant cutaneous acute radiation syndrome and G3 refers to significant gastrointestinal ARS per the “Fleider Protocol”).*
- 4. What are the minimum logistical requirements for medical care of radiation exposure patients?**
  - a. Expected recovery with observation and modest support*
    - i) Equivalent to H1 diagnosis (Degree 1 in table below)*
  - b. Probable recovery with intermediate support*
    - i) Equivalent to H2 diagnosis (Degree 2 in table below)*
  - c. Possible recovery with intensive support including transplantation of HPC*
    - i) Equivalent to H3/H4 diagnosis (Degree 3 & 4 in table below)*

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Table 1. Acute Radiation Syndrome Severity Rating by Laboratory Tests for the Hematopoietic Subsyndrome**

| HEMATOPOIETIC SUBSYNDROME <sup>1</sup>                                                                                                                                                                                                                                                        |          |                            |                                                                                 |                             |
|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|----------|----------------------------|---------------------------------------------------------------------------------|-----------------------------|
| Sign/Symptom/Test                                                                                                                                                                                                                                                                             | Degree 1 | Degree 2                   | Degree 3                                                                        | Degree 4                    |
| <b>24 - 48 HOURS</b> Serial CBCs recommended to improve estimation of severity. (Lymphocyte kinetics and dose, How frequent?)                                                                                                                                                                 |          |                            |                                                                                 |                             |
| Lymphocyte count (10 <sup>9</sup> cells/L)                                                                                                                                                                                                                                                    | ≥ 1.5    | 1.5 - 1                    | 1 - 0.5                                                                         | < 0.5                       |
| Granulocyte count (10 <sup>9</sup> cells/L)                                                                                                                                                                                                                                                   | > 2      | 4 - 6, mild granulocytosis | 6 - 10, moderate granulocytosis                                                 | > 10, marked granulocytosis |
| Platelet count (10 <sup>9</sup> cells/L)                                                                                                                                                                                                                                                      | ≥ 100    | 100 - 50                   | 100 - 50                                                                        | 100 - 50                    |
| <b>3 - 7 DAYS</b> Serial CBCs recommended to improve estimation of severity. (Lymphocyte kinetics and dose, How frequent?)                                                                                                                                                                    |          |                            |                                                                                 |                             |
| Lymphocyte count (10 <sup>9</sup> cells/L)                                                                                                                                                                                                                                                    | ≥ 1      | 1 - 0.5                    | 0.5 - 0.1                                                                       | < 0.1                       |
| Granulocyte count (10 <sup>9</sup> cells/L)                                                                                                                                                                                                                                                   | > 2      | > 2                        | > 5<br>(note the high granulocyte with low platelets is a poor prognostic sign) | > 5                         |
| Platelet count (10 <sup>9</sup> cells/L)                                                                                                                                                                                                                                                      | ≥ 100    | 100 - 50                   | 50 - 20                                                                         | < 20                        |
| <small>1. Modified from the NIH REMM draft ARS treatment website, which created the original by modifying from Fliedner, TM, Friesecke, I, Beyrer K. Medical Management of Radiation Accidents: Manual on the Acute Radiation Syndrome. Oxford: British Institute of Radiology; 2001.</small> |          |                            |                                                                                 |                             |

5. **What is the expected availability of REAC/TS staff to answer questions from RITN centers on internal contamination, etc...?**
  - a. *REAC/TS will have staff at office in TN as well as one or two teams with the Federal Radiological Monitoring and Assessment Center (FRMAC).*
  - b. *REAC/TS staff will be accessible to answer treatment questions at a minimum via email.*
  - c. *Staff in TN will be able to ship decorporation agents.*
6. **What are the criteria for determining which patients are appropriate candidates for HLA typing, donor recruitment and transplantation?** With the intent that 1) resources not be wasted on candidates who are not appropriate and 2) that the transplantation process be expedited for those who are appropriate candidates.
  - a) **Typing- Perform HLA typing if expected to survive other injuries AND one or more of the following:**
    - a. *Perform HLA typing if whole body dose equivalent to >3 Gy*
    - b. *Neutrophil <100, beyond 7 days from exposure*
    - c. *Rapid drop in platelet, consistent with H3 or H4*
  - b) **Recruitment- Recruit donor if expected to survive other injuries AND ALL of the following:**
    - a. *Bone marrow is aplastic at two locations (Day 14-D21 after exposure)*
    - b. *Neutrophil sill <100 after 5 days of cytokines*
    - c. *Suitable donor:*
      - i. *8/8 match (HLA-A, B, C, DRB1) for bone marrow or PBSCs*
      - ii. *4/6 match for cord blood*

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

*iii. Haploidentical or mismatched T-cell depleted transplant would be an alternative is available*

**c) Infusing Cells – Infuse cells if expected to survive other injuries AND ALL of the following:**

- a. Aplastic bone marrow*
- b. ANC <100*
- c. Beyond Day 21 after exposure and received 7-10 days of GCSF*



## Acute Radiation Syndrome Treatment Guidelines

August 2012

Please forward comments or suggestions to [RITN@nmdp.org](mailto:RITN@nmdp.org)

NMDP systems were enhanced during the period of performance. Additional data storage capacity was procured to allow:

- Synchronization of critical data for Donors and Cords
- Continued support for the disaster recovery information technology environment

These enhancements will reduce the time to resume critical operations and ensure limited to no interruptions in access to search and procurement services.

### **Aim A.1.4: National Data Collection Model**

No funding was requested under this Aim for the 0339 budget cycle.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.A. Contingency Preparedness – Hypothesis 2:**

Coordination of the care of casualties who will require hematopoietic support will be essential in a contingency situation.

### **Aim A.2.1: Contingency Response Network**

This Aim focuses on the activities of the RITN. The RITN was organized to provide comprehensive evaluation and treatment for victims of radiation exposure or other marrow toxic injuries. The RITN develops treatment guidelines, educates health care professionals, works to expand the network, and coordinates situation response. The RITN is a cooperative effort of the NMDP and The American Society for Blood and Marrow Transplantation (ASBMT).

During this period of performance, the RITN grew to include 60 centers comprised of:

- 47 transplant centers
- 6 donor centers
- 7 cord blood banks

The RITN provides comprehensive evaluation and treatment for victims of radiation exposure or other marrow toxic injuries.

Many of the casualties with radiation injury will be salvageable but require outpatient and/or inpatient care. Recognizing this, the NMDP, US Navy, and American Society of Blood and Marrow Transplantation (ASBMT) collaboratively developed RITN, which is comprised of medical centers with expertise in the management of bone marrow failure, stem cell donor centers, and umbilical cord blood banks across the US.

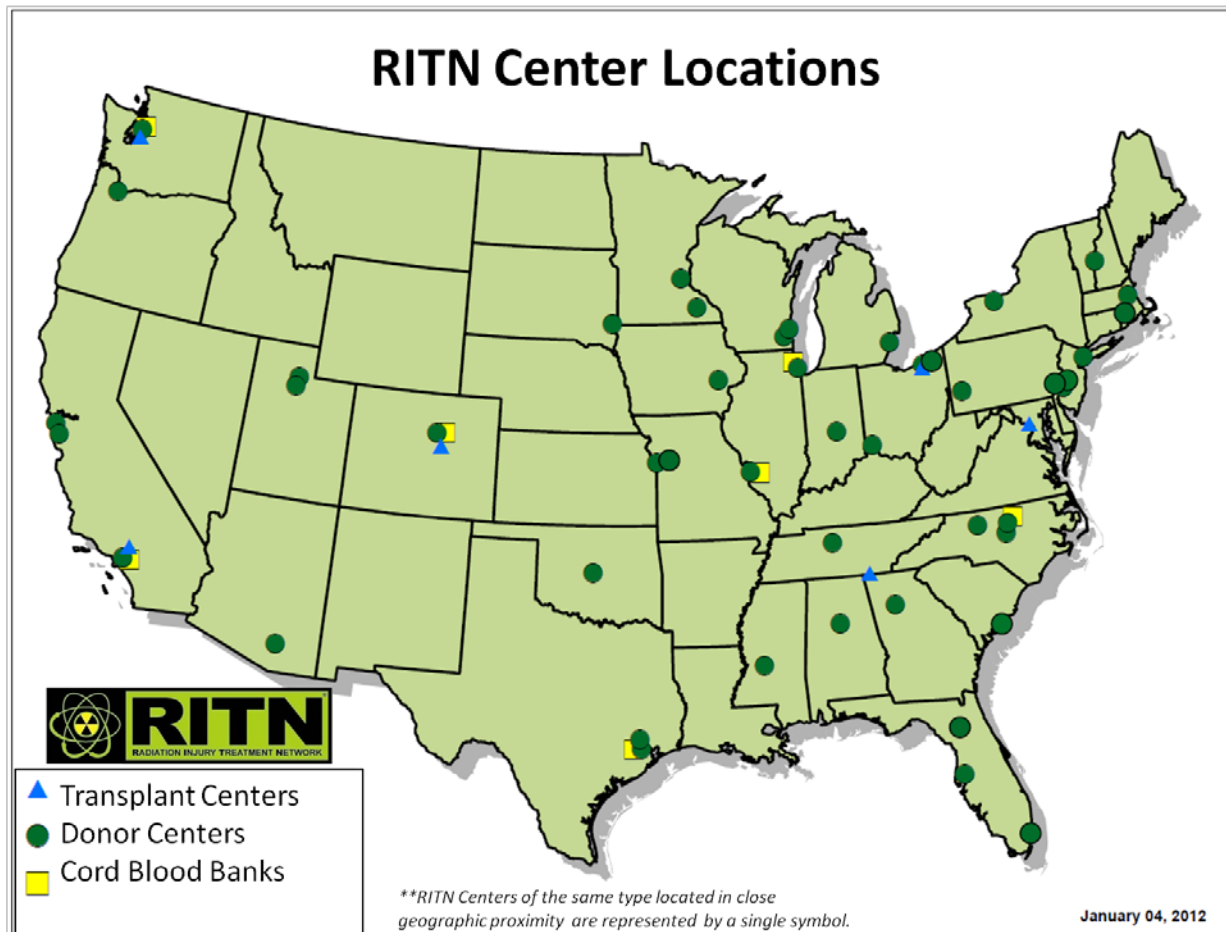
The goals of RITN are:

1. To develop treatment guidelines for managing hematologic toxicity among victims of radiation exposure,
2. To educate health care professionals about pertinent aspects of radiation exposure management,
3. To help coordinate the medical response to radiation events, and
4. To provide comprehensive evaluation and treatment for victims at participating centers.

These centers are spread across the nation as shown in the Figure 3 below.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

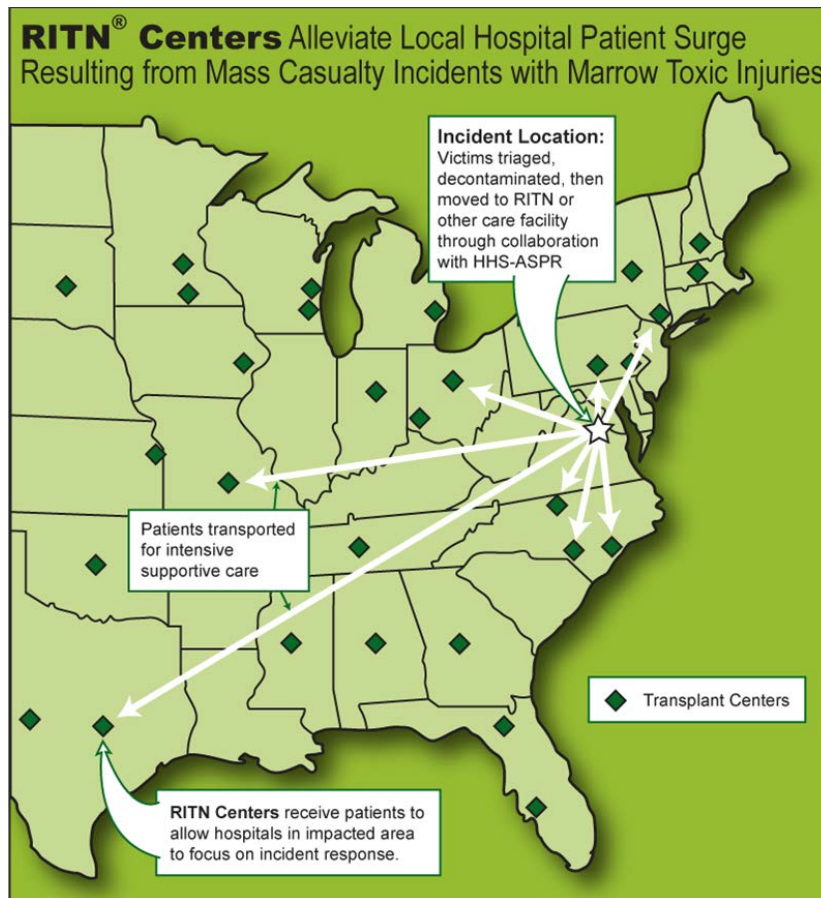
**Figure 3. RITN Center Locations in the United States**



RITN centers are prepared to receive casualties from a mass casualty incident with marrow toxic injuries; however, it is important to understand that RITN centers are not first responders. All participating centers voluntarily prepare to respond to an incident that occurs in another city or some other distant location. The NMDP anticipates that the RITN will receive patients from another part of the country to alleviate their medical load and to provide the best care possible (see figure below for overview of casualty flow).

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**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 4. Overview of RITN Casualty Flow**

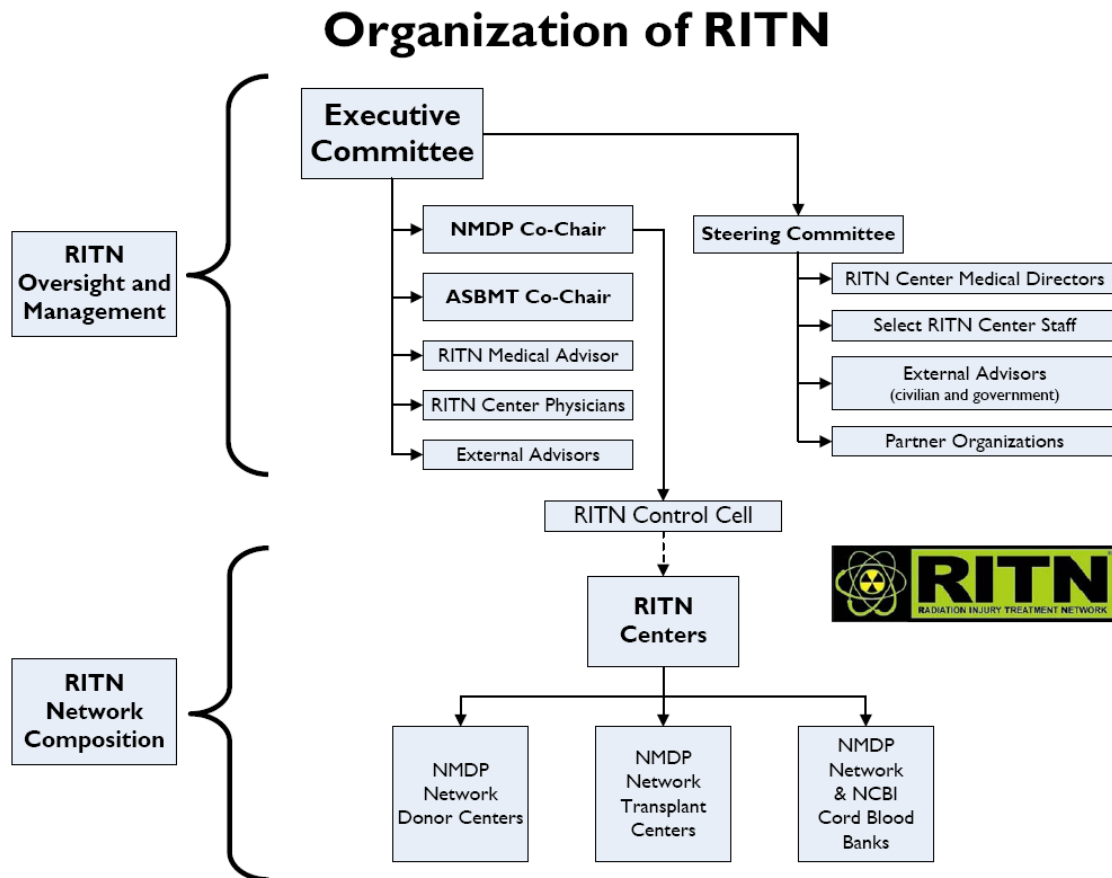


The RITN is managed by an Executive Committee which develops or reviews all RITN materials from the training courses to the treatment guidelines. As part of the Executive Committee, the RITN Control Cell interfaces with the network of medical professionals that RITN is comprised of. Supporting the Executive Committee is a Steering Committee consisting of RITN center staff, federal advisors, and partners.

The Executive Committee meets every other month via teleconference and the Steering Committee meets once a year during the annual ASBMT/CIBMTR Tandem BMT Meetings. Since many members of RITN already regularly attend this annual conference, there is a cost savings to hold the Steering Committee meeting at the conference as well as broadcasting the strong relationship between the RITN and ASBMT.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 5. Organization of RITN**



The **RITN Executive Committee** is co-chaired by a representative from the NMDP and from the ASBMT, and assisted by a Medical Advisor, other physicians, and technical advisors that support the activities of this committee:

- Committee Chairs:
  - o Co-Chair: Dennis Confer, MD
  - o Co-Chair: Nelson Chao, MD (ASBMT past President)
- RITN Medical Advisor:
  - o David Weinstock, MD
- Committee Members:
  - o Transplant Physician: Daniel Weisdorf, MD (ASBMT past President)
  - o Transplant Physician: John Chute, MD
  - o ASBMT Representative: Julie Wilhauk, ARNP, AOCNP
  - o ASBMT Representative: Robert Krawisz, MBA
  - o RITN Program Manager: Cullen Case Jr., CEM

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Some of the outcomes of Executive Committee conference calls held during this period of performance include:

- Identification of possible bone marrow transplant programs to be invited to join RITN in the future
- Development of the agenda, content, and speakers for the 2011 RITN Educational Conference “State of the Science Workshop: Radiation Exposure, Medical Countermeasures and Treatment”
- Development of agendas for Steering Committee meetings and coordination of presentations by external subject matter experts at the Steering Committee meetings

A key partnership was initiated with the American Hospital Association (AHA), National Association of City and County Health Officials (NACCHO), and the Association of State and Territorial Health Officials (ASTHO).

During the period of performance, the **Steering Committee** held three in person meetings:

- February 2011 - ASBMT/CIBMTR Tandem Meetings (Honolulu, HI)
- October 2011 – 2011 RITN Educational Conference (Chicago, IL)
- February 2012 - ASBMT/CIBMTR Tandem Meetings (San Diego, CA)

Monthly, all RITN center staff, as well as RITN partners, are invited to a conference call where updates are provided on current projects, RITN center staff are afforded an opportunity to talk about implementation issues with other more seasoned RITN centers, and the “Rad in the News” is reviewed. “Rad in the News” is a summary of highlights with links to open source media reports about radiological related current events. In addition to these monthly meetings, each December, a RITN Year in Review webinar was held which reviews the accomplishments during the year and planned activity in the upcoming year.

### **RITN Annual Tasks**

RITN centers are tasked each year with completing a set of tasks in exchange for a small grant. During this period of performance, centers had to update their standard operating procedures, conduct a tabletop exercise, and conduct training of staff.

**Table 2. Summary of Tasks for RITN Centers by type**

| <b>TASK SUMMARY TABLE:</b> |               |               |               |               |               |               |               |              |
|----------------------------|---------------|---------------|---------------|---------------|---------------|---------------|---------------|--------------|
|                            | <b>Task 1</b> | <b>Task 2</b> | <b>Task 3</b> | <b>Task 4</b> | <b>Task 5</b> | <b>Task 6</b> | <b>Task 7</b> | <b>Grant</b> |
|                            | Contact       | SOP           | Tabletop      | Commo.        | Overview      | Educate       | Site Eval.    |              |
| <b>TC</b>                  | Yes           | Yes           | Yes           | Yes           | Yes           | 6 or 7        | 6 or 7        | \$8,000      |
| <b>DC</b>                  | Yes           | Yes           | No            | Yes           | 5 or 6        | 5 or 6        | No            | \$2,000      |
| <b>CBB</b>                 | Yes           | Yes           | No            | Yes           | 5 or 6        | 5 or 6        | No            | \$2,000      |

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Annual RITN Tabletop Exercise (TTX)**

Each year, one of the tasks is to conduct a tabletop exercise with hospital staff and local public health preparedness officials. During this period of performance, the scenario for the tabletop exercise focused on the flow of casualties through the National Disaster Medical System in response to the detonation of a 10kT improvised nuclear device:

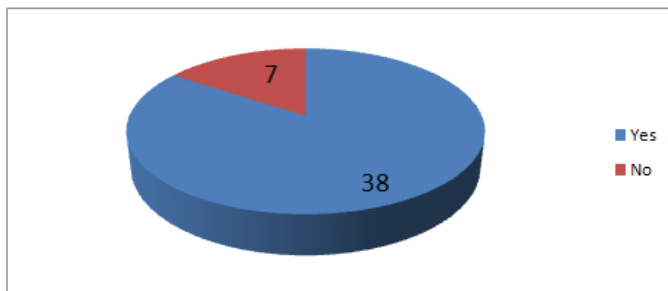
*A mass casualty incident that results in marrow toxic injuries most likely would result from a terrorist detonation of an improvised nuclear device or a catastrophic industrial accident. The Department of Homeland Security's National Planning Scenarios include 16 possible terrorist incidents that would significantly impact the United States. Each scenario describes the scope of the incident and offers estimates of the number of resulting casualties. One of these scenarios is the detonation of a 10 kT (kiloton) improvised nuclear device, which is approximately equivalent to the bomb detonated over Hiroshima during World War II.*

Each RITN center was asked at the conclusion of the exercise how they would respond to a request to potentially accept up to 200 casualties. The results of each year's tabletop exercise, including each centers response to all of the questions, are posted (in a non-attributable format) on the RITN website: [.RITN.net/exercises/](http://RITN.net/exercises/). Some highlights of responses from this year's exercise are included below.

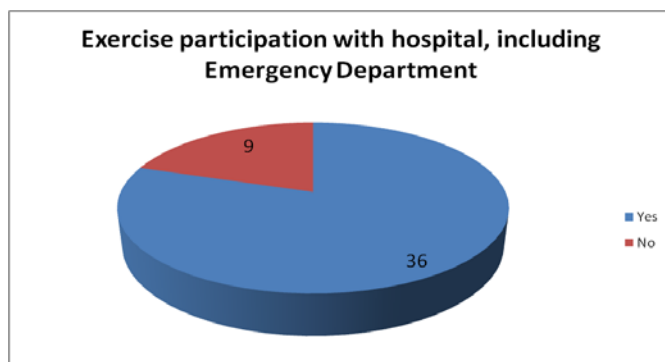
**Figure 6. Selected Responses from RITN Tabletop Exercise Questions**

**Figure 6a.**

**Is your hospital contracted with the National Disaster Medical System?**

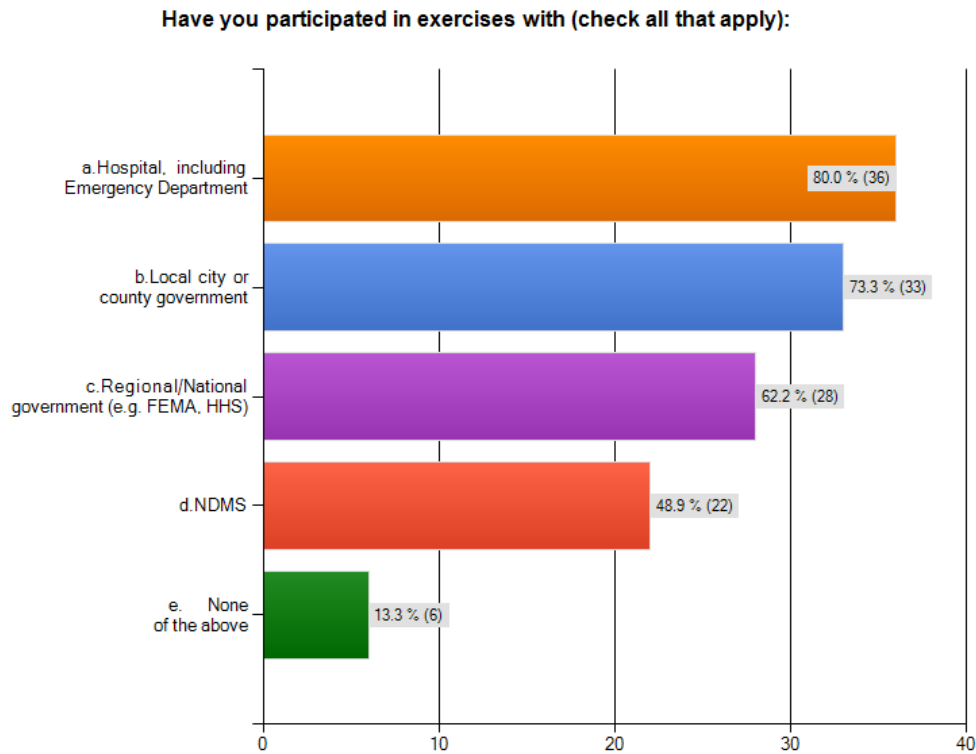


**Figure 6b.**



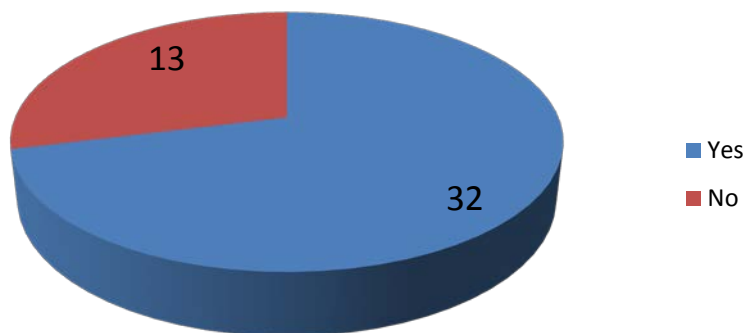
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**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 6c.**



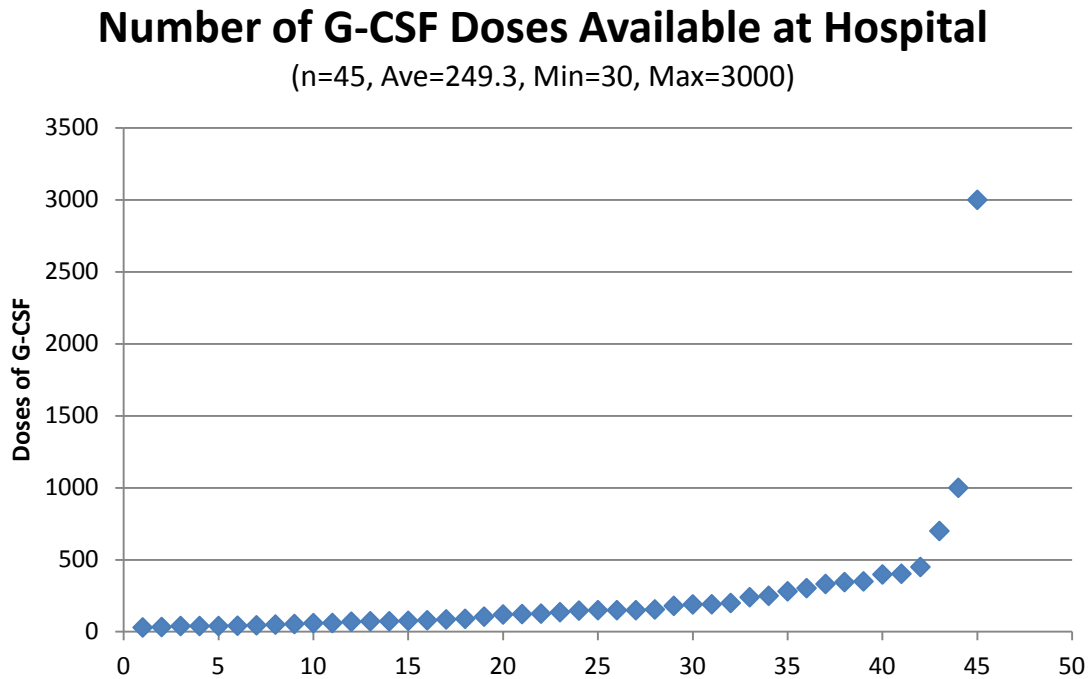
**Figure 6d.**

**Are there particular medications (e.g. antibiotics), supplies (e.g. IV tubing, blood products), or equipment (e.g. beds, IV pumps) that you anticipate would be in short supply at your center and therefore limit your ability to care for a large number of irradiated casualties?**



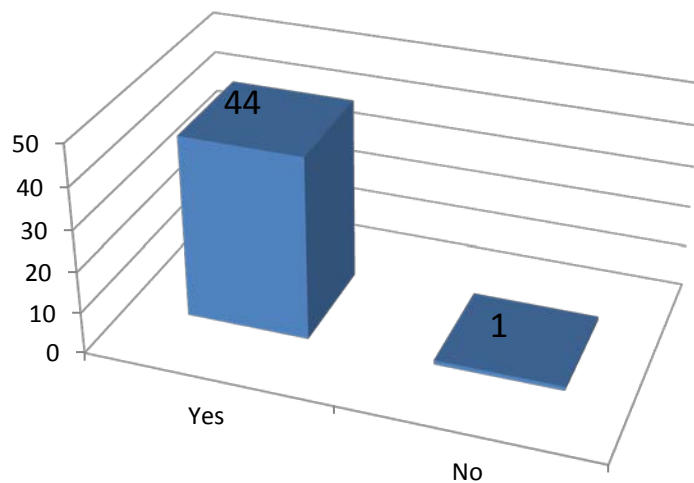
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**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 6e.**



**Figure 6f.**

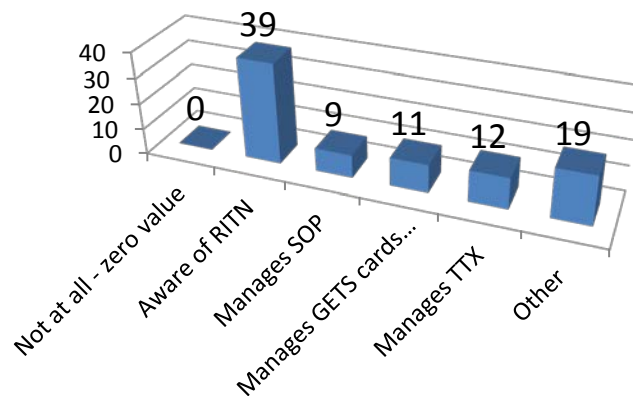
**Facilities that have plans for housing family members who arrive with injured casualties?**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 6g.**

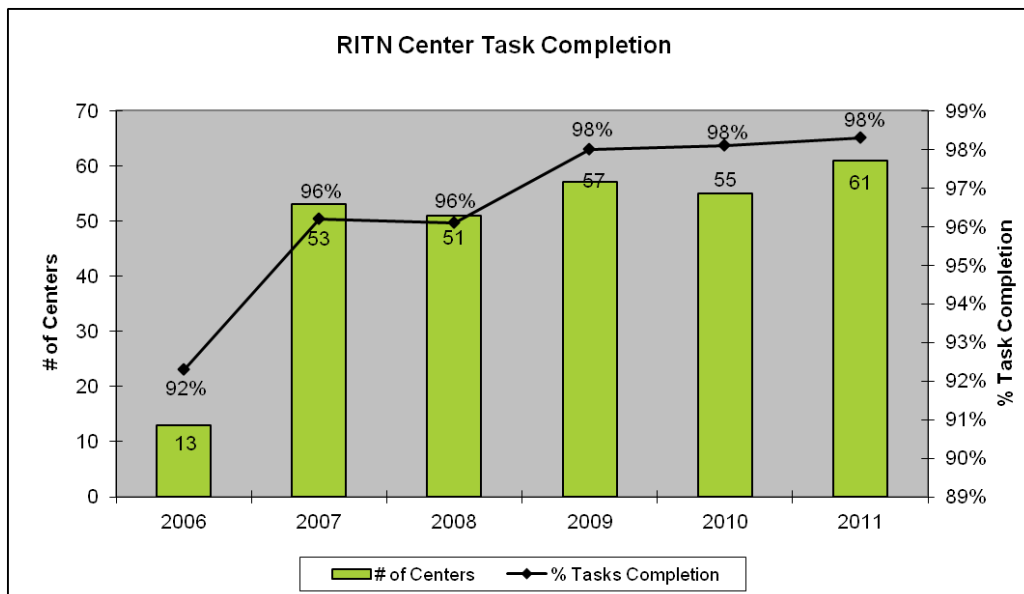
**To what extent is your hospital emergency manager/coordinator involved with RITN?**



### Annual Task Completion

During this grant period, 98% of the RITN centers completed all of their tasks (Figure 7), a slight improvement over the previous grant period.

**Figure 7. RITN Center Task Completion**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**RITN Partnerships**

The RITN would not be able to successfully respond to a mass casualty incident without the support of partner organizations. The NMDP has carefully worked to develop and maintain these relationships so that when an event occurs, established relationships are already in place with these key response organizations.

RITN has two types of relationships; formal relationships are documented through a Memorandum of Understanding (MOU) and informal relationships through periodic collaboration.

RITN has established formal partnerships through an MOU with:

- Office of the Assistant Secretary for Preparedness and Response, Department of Health and Human Services (ASPR-DHHS)
- American Society for Blood and Marrow Transplantation (ASBMT)
- American Association of Blood Banks (AABB), through the AABB Inter-organizational Task Force for Disasters and Acts of Terrorism
- New England Center for Emergency Preparedness (NECEP)

Organizations that RITN has developed informal relationships with include:

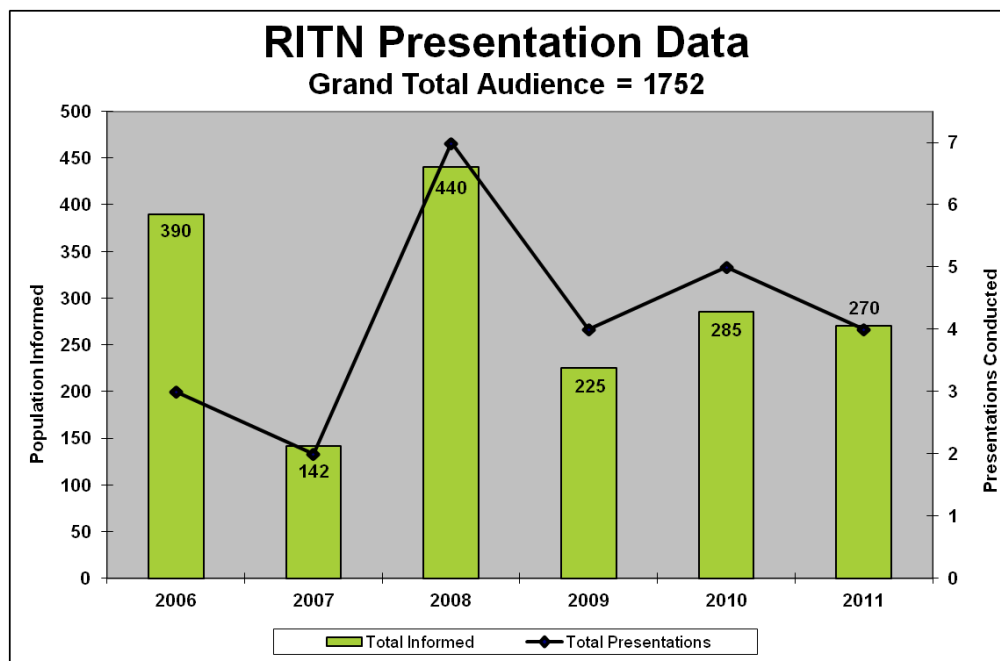
- Radiation Countermeasures Centers of Research Excellence (RadCCORE) at Duke University
- The National Institutes of Health, The National Institute of Allergy and Infectious Diseases, Division of Allergy, Immunology and Transplantation (NIH-NAIAD-DAIT)
- Radiation Emergency Medical Management web portal (NIH-NLM-REMM)
- National Cancer Institute's (NCI)
- Biomedical Advanced Research and Development Authority (BARDA)
- European Group for Blood and Marrow Transplantation (EBMT) - Nuclear Accident Committee
- The Radiation Emergency Medical Preparedness and Assistance Network of the World Health Organization (WHO-REMPAN)
- Radiation Emergency Assistance Center and Training Site (REAC/TS)
- American Hospital Association (AHA)
- American Medical Association (AMA)
- National Association of City and County Health Officials (NACCHO)
- Association of State and Territorial Health Officials (ASTHO)

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Education and Awareness Training about RITN**

To increase the visibility of RITN and make new connections with additional organizations and agencies, overview presentations were given to various professional groups and government agencies. The chart below summarizes the number of these presentations given, as well as the size of the audience affected:

**Figure 8. RITN Presentation Data**

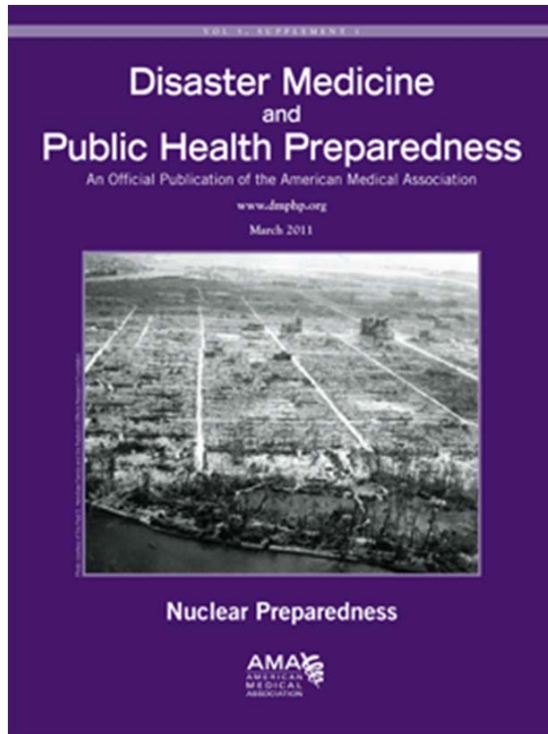


**Publications**

Another avenue of increasing visibility is through publication in peer reviewed journals. The Executive Committee was involved with many publications during this period of performance; however, the most visible and impactful to the preparedness of the nation is the “Disaster Medicine and Public Health Preparedness Nuclear Preparedness” journal in which the RITN Medical Advisor co-authored 8 of the 17 articles (the journal is open source and available online: [http://www.dmphp.org/content/vol5/Supplement\\_1/](http://www.dmphp.org/content/vol5/Supplement_1/))

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 9. RITN Publication from March 2011**



Additional publications include:

- N Dainiak, RN Gent, Z Carr, et al. Literature Review and Global Consensus on Management of Acute Radiation Syndrome Affecting Non-Hematopoietic Organ Systems, Disaster Medicine and Public Health Preparedness, October 2011
- N Dainiak, RN Gent, Z Carr, et al. First Global Consensus for Evidence-Based Management of the Hematopoietic Syndrome Resulting from Exposure to Ionizing Radiation, Disaster Medicine and Public Health Preparedness, October 2011
- Weisdorf D, Weinstock D, Case C, Chao N, Confer DL. Planning and response to radiation exposures. Biol Blood Marrow Transplant. 2011 Aug;17(8):1262-3. Epub 2011 Jun 15.
- Joel Ross, Cullen Case, Nelson Chao, et al. Radiation Injury Treatment Network (RITN): Healthcare professionals preparing for a mass casualty radiological or nuclear incident. Int. J. Radiat. Biol., Vol. 87, No. 5, PrePub Feb 2011

For the 104 days following the disaster at the Japanese Nuclear Power Plant in Fukushima, daily summary situational awareness reports were sent to RITN centers and RITN partner organizations. Requests from the AHA to the CDC were made to receive copies of these reports, increasing the number of RITN partners and RITN's visibility in the preparedness community.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Many radiological or nuclear disaster articles were written that highlighted the incident in Japan. The following articles referenced RITN or quoted RITN Executive Committee members or staff from RITN centers:

1. 4/15/2011 - Should Japan Bank Stem Cells From Fukushima Nuclear Workers? Science, Accessed 4/18/2011, [://news.sciencemag.org/scienceinsider/2011/04/should-japan-bank-stem-cells.html?ref=](http://news.sciencemag.org/scienceinsider/2011/04/should-japan-bank-stem-cells.html?ref=) (Weinstock-RITN Med. Advisor, Chao-RITN Exec. Cmte)
2. 4/10/2011 - Transplants for Thousands, New York Times, Accessed 4/15/2011, [://www.nytimes.com/2011/04/11/opinion/lweb11marrow](http://www.nytimes.com/2011/04/11/opinion/lweb11marrow). (Chell-NMDP CEO)
  - a. In response to the 4/1/2011 article New Urgency in Push for Radiation Drugs [://www.nytimes.com/2011/04/01/business/01radiation.html?\\_r=](http://www.nytimes.com/2011/04/01/business/01radiation.html?_r=)
3. 3/29/2011 - Amid Japan crisis, hunt for better radiation care, Associated Press, Accessed 4/18/2011, [://www.google.com/hostednews/ap/article/ALeqM5gWg0vQLaVibv2GDZ9Hs5JVYQ91ug?docId=](http://www.google.com/hostednews/ap/article/ALeqM5gWg0vQLaVibv2GDZ9Hs5JVYQ91ug?docId=) (Chao-RITN Exec. Cmte)
4. 3/17/2011 - As Radiation Drifts Toward U.S., Officials Downplay Risk, San Gabriel Valley Tribune, Accessed 3/18/2011, [://www.sgvtribune.com/news/ci\\_17638624?IADID=Search-www.sgvtribune.com-www.sgvtribune](http://www.sgvtribune.com/news/ci_17638624?IADID=Search-www.sgvtribune.com-www.sgvtribune). (Chuck Pickering-COH, LA)
5. 3/17/2011 - Little Protection for Those on the Front Lines, Science, Accessed 3/18/2011, [://news.sciencemag.org/scienceinsider/2011/03/little-protection-for-those-on-t.html?ref=](http://news.sciencemag.org/scienceinsider/2011/03/little-protection-for-those-on-t.html?ref=) (Weinstock-RITN Med. Advisor, Chao-RITN Exec. Cmte)
6. 3/15/2011 - Experts plan for how to deal with nuclear terror strike, USA Today, Accessed 3/17/2011, [://www.usatoday.com/news/nation/2011-03-15-nukemed14\\_ST\\_N.htm?loc=](http://www.usatoday.com/news/nation/2011-03-15-nukemed14_ST_N.htm?loc=) (Weinstock-RITN Med. Advisor)

**Aim A.2.2: Develop and test standard operating procedures, in conjunction with core transplant centers, to manage the activities required to HLA type siblings of casualties to evaluate their potential as HSC donors for their affected family member.**

No funding was requested under this Aim for the 0339 budget cycle.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.A. Contingency Preparedness – Hypothesis 3:**

NMDP's critical information technology infrastructure must remain operational during contingency situations that directly affect the Coordinating Center.

**Aim A.3.1: Disaster Recovery: Ensure NMDP's ability to access and utilize its information management and communication infrastructure in a contingency situation in which its Minneapolis Coordinating Center is damaged or destroyed.**

No activity was requested under this Aim for the 0339 Budget Cycle

### **Aim A.3.2: Operational Continuity Planning:**

The focus of this Aim is to improve organizational resiliency to severe operational disruptions through Operational Continuity Planning. In the event that the Coordinating Center is not available for an extended period of time, critical tasks will have to be conducted at an alternate location. To meet these needs, the NMDP maintains an Operational Continuity Plan (OCP), consisting of documented procedures for guiding the organization to respond, recover, resume and restore a predefined level of operations following disruptions, including a Critical Task List to prioritize the response.

The OCP is annually reviewed by key stakeholders to test planned response actions with current business practices and predefined maximum tolerable periods of disruption (MTPD). MTPD is the time it takes for adverse impacts as a result of not providing services or performing activity to become unacceptable. Inputs for the OCP improvement include current operations impact analyses, functional and tabletop exercises, and industry regulatory or standard changes. The OCP is adaptable and enables the organization to respond to a wide range of disruptive incidents, including those the NMDP may not have anticipated. The NMDP's formal OCP is maintained by the Operational Continuity Planner.

During this period of performance the NMDP conducted several significant exercises. A tabletop exercise with the Operational Continuity Planner and the IT Disaster Recovery staff (OC/DR) focused on a step-by-step review of incident response flow for the following contingency incident scenarios:

- Coordinating Center unavailable, data center available
- Coordinating Center available, data center unavailable
- Both Coordinating Center and data center unavailable

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

This 2011 OC/DR exercise identified a number of action items, most of which were resolved during the contract period:

- Lack of a defined off-site software repository with current production versions available: **Resolved in 2012**
- Lack of VPN capacity to service 175 identified operational continuity recovery staff remotely: **Resolved in 2012**
- Single point of failure with all Minnesota facilities connecting as spokes directly to the Broadway Ridge facility hub with only Broadway Ridge connecting to the data center: **Resolved in 2012**
- The lack of dedicated contracted operational continuity recovery sites makes staff recovery capacity uncertain since potential sites are selected based on availability at time of disaster: **Issue acknowledged; leasing and outfitting fixed recovery sites is cost prohibitive to implement.**
- Operational recovery following regional disasters impacting both the Coordinating Center and the data center will take at least six weeks, possibly longer. Currently there is no plan to recover in a timelier manner from this scenario until an alternate or backup data center is established outside of the Twin Cities: **Issue acknowledged, cost prohibitive to implement.**

During the 2011 operational continuity exercise, 100 tasks were attempted by a total of 68 operational continuity recovery staff, 86% of tasks were successfully accomplished. Participating operations units included: Quality Assurance, Legal and Risk Management, Case Management, Human Resources, Patient Services, Donor Liaison Team, Public Engagement Team, Finance, Survey Research Group, Scientific Services, and the Center for Cord Blood.

The 2011 Operational Continuity Exercise successfully met these exercise objectives:

- Establish secure data center connectivity from an ad hoc Critical Staff Recovery Site.
- Establish Unified Communications (IP phones) at ad hoc Critical Staff Recovery Site.
- Assess ability of participating department staff to complete critical tasks.

This 2011 operational continuity exercise identified a number of action items, most were resolved during the contract period:

- Some of the terminal servers lacked the necessary links and programs needed for some departmental user access testing: **Resolved 2012**
- Some users were reliant on remote access to desktop systems that may be unavailable during a severe business interruption to complete critical tasks: **Resolved 2012**
- HR required access to a specific virtual drive (M drive) that could not be accessed remotely to execute payroll and benefits transactions: **Resolved 2012**
- Check writing capacity requires specialized software, data files, and equipment to execute not available at recovery site: **Resolved 2012**

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

To support the establishment of additional Operational Continuity Recovery Sites, supplemental long lead time equipment was purchased. This equipment is stored at the Repository Facility in New Brighton, Minnesota. Equipment purchases included powered VPN appliances, high capacity powered switches, wireless access points, and headsets for software phones.

To sustain communications with Network partners during a severe operational disruption, the NMDP maintains a variety of redundant channels. The NMDP has over 150 active Governmental Emergency Telecommunications Service (GETS) emergency calling cards issued to RITN centers and NMDP staff and over 60 Iridium satellite telephones assigned and distributed to external partners. Recurring tests of each of these capabilities ensured user familiarity and equipment accountability.

Site visits to several NMDP operated donor centers resulted in improved preparedness for NMDP field staff. A review of the NMDP Operational Continuity Action Guide with each site manager and their staff ensured they know what to do for the major hazards applicable to their location. Processes for closing offices due to local hazards and transferring critical activity to other facilities were refined.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.B. Rapid Identification of Matched Donors – Hypothesis 1:**

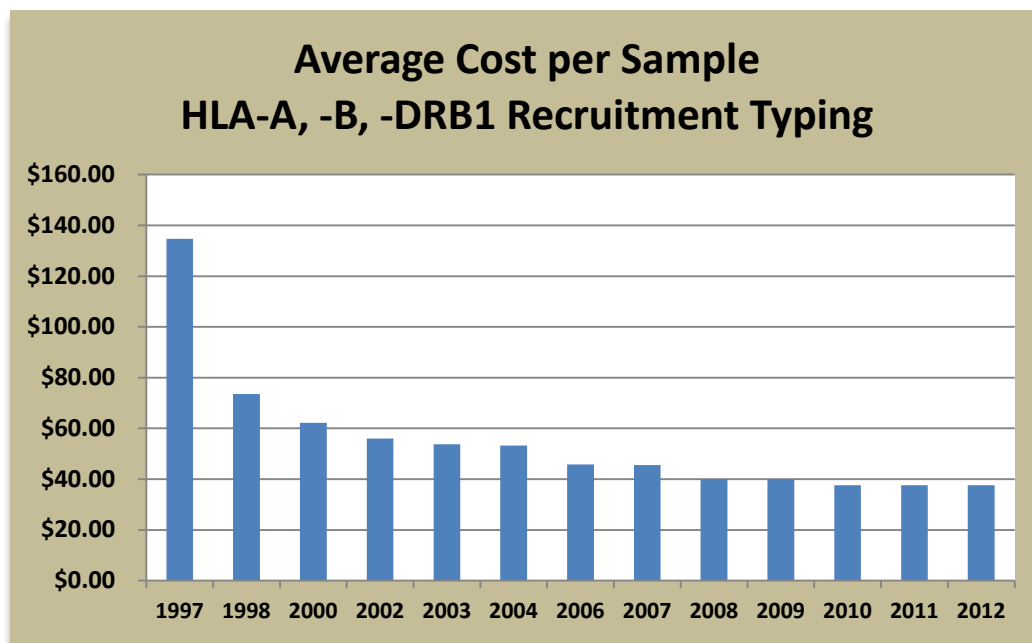
Increasing the resolution and quality of the HLA testing of volunteers on the registry will speed donor selection.

Advances in laboratory methods and supporting equipment continued to positively impact the level of quality and typing resolution for newly recruited volunteer donors. As of December 31, 2012, all newly recruited donors were being typed at a minimum of 3 loci (HLA-A, B and DRB1). 52% of these donors were being typed at 5 loci (adding HLA-C and DQB1), and 18% were being typed at 6 loci (adding HLA-C, DQB1 and DPB1). Furthermore, 41% of all donors were typed at laboratories used Sequenced Based Typing (SBT)

In an effort to maximize these resources and ensure the optimal use of typing funds, a process was implemented that allows selective typing based on donor characteristics. Samples from donors with particularly desirable demographics (male, younger age and minority) are directed to specific laboratories to ensure they are listed on the registry with the best typing possible.

Over the past 15 years, the NMDP has reduced the cost of HLA typing by over 70% while increasing typing resolution and quality (Figure 10). The vision and efforts of the Navy project officer to continually press the HLA community to lower costs and increase typing resolution and quality has been instrumental in achieving these accomplishments.

**Figure 10. Cost Decreases for HLA-ABDR Typing**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Average cost per sample for HLA-A, B, DRB1 recruitment typing has decreased over time, from more than \$130/sample to less than \$40/sample. While per sample costs have been relatively constant over recent years, it should be noted that the resolution of the results has continued to increase over time, thereby facilitating improvements in donor selection without increased cost.

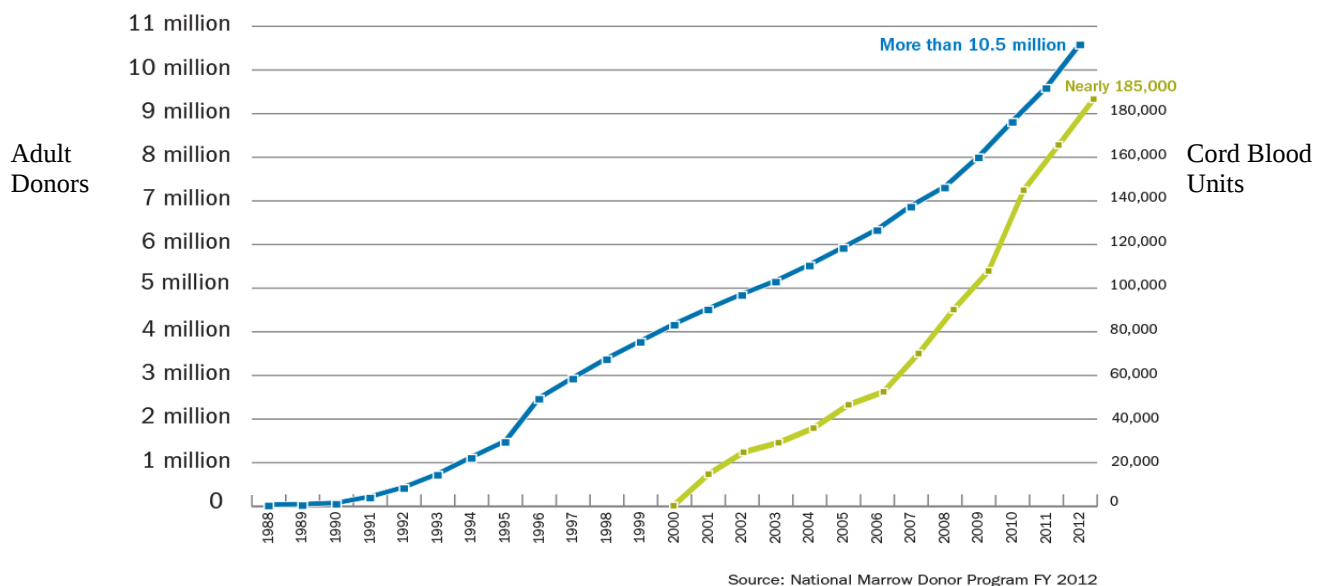
If a patient does not find a matched donor and is in urgent need, patient-focused drives can be held and the donor registration process can be expedited, shortening the length of time to listing from 6-8 weeks to 3 weeks. This process includes time to enter demographic data, confirm financial coverage, ship and receive the samples and complete the HLA typing. Demographic data are entered within 72 hours for expedited samples, and they are shipped the next scheduled day, Monday through Thursday. In case of a contingency event, high volumes of samples could be processed and shipped quickly using this established process.

### **Aim B.1.1: Increase Registry Diversity**

#### **Newly recruited donors increased diversity**

During NMDP FY11, NMDP donor centers (including DoD) and recruitment groups recruited 259,612 minority race and 249,104 Caucasian donors, for a total of 508,716 U.S. donors added to the Registry. Navy funding contributed to the addition of 143,280 of this culturally diverse group of new donors, with 46,834 of these donors being minorities. All donors were typed for a minimum of HLA-A, B, and DRB1.

**Figure 11. Be the Match Registry Growth: Adult Donors and Cord Blood Units**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**New Donor Queue Sorting**

To maximize the utilization of the HLA recruitment typing resources, a process was developed to strategically select samples from the newly recruited donor queue and direct these samples to specific laboratories. This process allows the NMDP to select donors, based on demographic data, and direct the testing to laboratories that provide the most complete and highest resolution HLA typing at the time of recruitment. The goal is to select the most valuable donors (young males, young females, and all minorities) and ensure they are listed on the registry with the most comprehensive typing available through the contract laboratory network.

This queue sorting process has provided a dramatic increase in the number of loci typed on the most valuable new donors joining the registry, which are male (M) and female (F) donors, 18-30 years of age. The below information shows the gender recruited with associated HLA typing:

|                |     |     |                               |
|----------------|-----|-----|-------------------------------|
| January 2011:  | 95% | M&F | HLA-A, B, C, DRB1             |
| December 2012: | 23% | M   | HLA-A, B, C, DRB1, DQB1       |
|                | 77% | M   | HLA-A, B, C, DRB1, DQB1, DPB1 |
|                | 83% | F   | HLA-A, B, C, DRB1, DQB1       |
|                | 17% | F   | HLA-A, B, DRB1                |

**Advancing technology improved performance and pricing**

Advances in laboratory methods and supporting equipment continue to have a positive impact on lab performance and pricing.

As of December, 2012:

- 97% of new donors received higher than intermediate HLA-A, B and DRB1 typing
- 52% of new donors had additional loci tested (HLA-C, DQB1 and DPB1)
- Blind quality control testing error rate was 0.08%, exceeding the project requirement of  $\leq 2.0\%$ .
- On-time testing completion rate was 99%, meeting the project requirement of a minimum of 90% of typing results reported within 14 days of shipment of samples.
- The cost of HLA typing continued to decrease as technology improved; in December 2012, the average price per sample was approximately \$40.00 compared to \$134.75 in 1997 (\$181.17 in 2012), which represents a decrease of over 78%. Note that in addition to decreasing average cost, there were increases in the number of loci typed and resolution of results over this time period.

**HLA Typing Laboratory Meetings**

NMDP hosted individual meetings for representatives from all of the new member recruitment HLA typing laboratories to discuss operational topics including: the current scope of work,

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

future goals for registry HLA typing, and the laboratory's future HLA testing vision. These discussions were important to allow the NMDP to continue to provide low cost and high quality HLA typing for patients searching the registry. The consensus from the meetings was that the only immediate way to decrease costs of HLA typing was to increase the testing volume. In the longer term, laboratories are evaluating new and emerging technologies to decrease costs and increase resolution.

### **Quality of HLA Typing Improved**

The NMDP maintains lists of rare alleles as a service to the American Society for Histocompatibility & Immunogenetics (ASHI). These lists are derived from HLA allele level typings of patients, adult volunteers, and cord blood units in the Be The Match Registry. In an on-going Registry data quality project, adult volunteers with reported HLA typing containing a rare allele were evaluated to determine if the results were accurate based the following rules:

1. Sample assignments showed previous corrections
2. Samples typed >four years ago and allele not subsequently observed
3. Alleles reported in a sample whose race differed from the race the allele was initially described
4. Interpreted primary data received from the laboratory not consistent with assignment
5. Rare allele consistently reported as a pair with the same second allele
6. Sample carried two rare alleles at the same locus.

During this contract period, 479 samples were typed at high resolution HLA-A, B, C, or DRB1. The rate at which results changed was 44.1%. Results from this grant period are detailed in Table 3. The cumulative testing for this allele retyping project funded by consecutive ONR grants shows that 903 (60%) donor typings changed from the previously reported rare allele and 605 donor typings were confirmed to carry the reported rare allele.

**Table 3: Results of HLA retyping project**

|          | # Typed | % confirmed | % changed |
|----------|---------|-------------|-----------|
| HLA-A    | 105     | 57.1%       | 42.9%     |
| HLA-B    | 232     | 50.0%       | 50.0%     |
| HLA-C    | 25      | 28.0%       | 72.0%     |
| HLA-DRB1 | 117     | 72.6%       | 27.4%     |
| Total    | 479     | 55.9%       | 44.1%     |

The results of these data were presented at the International Histocompatibility and Immunogenetics Workshop/European Federation of Immunogenetics, "Frequency of Rare Alleles Workshop" in May 2012. A poster abstract detailing the inaccuracy of DRB1\*16:08 typing was also presented at this meeting.<sup>1</sup>

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Results of these re-typing projects improved the HLA typing quality of listed adult volunteers and removed erroneous typing from the registry. During this contract period, one adult volunteer whose typing had been corrected through this project was requested for additional testing on behalf of a searching patient. The re-typing project highlights the importance of technical oversight of the Registry data and the necessity to upgrade typings routinely in order to provide the most accurate HLA data for searching patients.

**Aim B.1.2: Evaluate HLA-DRB1 High Res Typing**

No funding was requested under this Aim for the 0339 budget cycle.

**Aim B.1.3: Evaluate HLA-C Typing of Donors**

No funding was requested under this Aim for the 0339 budget cycle.

**Aim B.1.4: Evaluate Suitability of Buccal Swabs**

Sample Storage Research Study

The 5-year Sample Storage Research Study began in September 2007. Samples from 30 fully HLA characterized volunteer quality control donors were collected, processed and stored at the NMDP Repository. The samples consisted of fresh blood, blood spotted onto Whatman® 903 filter paper and buccal swabs for each donor, were sent to two laboratories in September 2007 to initiate the study (Time Point Zero). One laboratory was contracted to perform high resolution typing for HLA-A, B, C, DRB1, and DQB1. The second laboratory was contracted to perform intermediate resolution typing for HLA-A, B, C, and DRB1 and to evaluate the quantity and quality of DNA derived from each sample type. Complete results were received from each of the two laboratories. All typing results were 100% accurate, and the evaluation of the DNA was complete and thorough. The results obtained at Time Point Zero are used as the baseline for comparison and evaluation of the stability and usefulness of the DNA stored in each sample type for the next 5 years. Results from this study will provide key quality parameters for NMDP business decisions concerning sample storage and may also contribute sample storage guidelines for other registries.

During this grant period, the results for the Time Point at 4 Years (funded in previous Navy 0204 contract) and at 5 Years (funded under this grant) were evaluated. In September, 2011, and September, 2012, 30 donor samples (frozen blood, blood spotted onto filter paper, and 2 buccal swabs for each donor) were sent to the two participating laboratories. A summary of the data is shown in Table 4 below.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Table 4. Sample Storage Research Study Results**

| <b>4 Year Time Point – 2011</b>                                                                                                                                                   | <b>5 Year Time Point – 2012</b>                                                                                                                                                                                                                   |
|-----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| HLA Typing: <ul style="list-style-type: none"> <li>• 100% accuracy for whole blood</li> <li>• 100% accuracy for filter paper</li> <li>• 100% accuracy for buccal swabs</li> </ul> | HLA Typing: <ul style="list-style-type: none"> <li>• 100% accuracy for whole blood</li> <li>• 99% accuracy for filter paper</li> <li>• 97% accuracy for buccal swabs</li> </ul>                                                                   |
| DNA Quantity: Sufficient for testing                                                                                                                                              | DNA Quantity: Sufficient for testing                                                                                                                                                                                                              |
| DNA Quality: Buccal swab DNA showing degradation <ul style="list-style-type: none"> <li>• 5 swabs needed repeat testing</li> </ul>                                                | DNA Quality: Filter paper samples showing degradation, Buccal swab DNA showing more degradation than in 2011 <ul style="list-style-type: none"> <li>• 14 filter papers needed repeat testing</li> <li>• 24 swabs needed repeat testing</li> </ul> |

Results show that DNA degradation issues first seen in Year 4 are tending to increase in Year 5, with buccal swabs showing degradation earlier than blood spotted onto filter paper. More details of the results will be published upon study completion. The NMDP is actively investigating alternative storage conditions that would provide greater long-term DNA stability for stored buccal swab samples, compared to the current controlled room temperature conditions. One option under consideration is storage of one buccal swab tip in a frozen state, for usage after long-term storage.

**Aim B.1.5: Enhancing HLA Data for Selected Donors**

No funding was requested under this Aim for the 0339 budget cycle.

**Aim B.1.6: Maintain a Quality Control Program**

The NMDP's comprehensive quality control program ensures the quality of HLA typing received through the contract laboratory network. In addition, this program helps to ensure the accuracy of data obtained from research studies that support abstracts and publications. Blind quality control (QC) samples are added to each weekly shipment of new donor recruitment samples. These QC samples comprise 2.5% of each shipment, and are indistinguishable from the donor samples. The Research Sample Repository contains frozen cells from thousands of fully HLA-characterized donors and recipients. QC swabs are created by the Repository staff from expanded B-Lymphocytic cell line (B-LCL) vials chosen from this resource. The immortalized B-LCL cells are applied to cotton-tipped swabs and included as QC in shipments of buccal swab donor samples.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

One hundred and sixteen samples were requested for cell transformation/initiation/culture/expansion from the NMDP Research Repository for incorporation into the B-LCL QC HLA typing program. Of these, 16 (14%) exhibited negative cell growth. A total of 100 unique buccal B-LCL QC Masters were added to the inventory, 91 of which were confirmatory typed at high resolution SBT prior to incorporation into the database. Six percent of high resolution confirmatory types were discrepant with previous high resolution types obtained from the research database. These 100 samples help ensure that the NMDP QC inventory has near comprehensive coverage the Common and Well-documented (CWD) common US alleles, and expand alleles that were depleted to an n of 1 to maintain desired allelic diversity.

The remaining budget was used to fund high resolution typing of 9 of the 20 cord blood units that were obtained at no cost from the NMDP Cord Blood Bank Network for inclusion into the cord QC program, which nearly doubled the total number of cord blood units available for cord QC purposes. These 9 units were the remaining units requiring typing, as 11 were previously HLA typed using Navy funds in 2007, grant 0859, Aim IIB.3.2, Enhancement of EM Algorithm. The cord QC program uses blood spots derived from cord blood units to mimic the material supplied to the confirmatory typing laboratory for the NMDP Cord Blood Bank Network.

## **II.B. Rapid Identification of Matched Donors – Hypothesis 2:**

Primary DNA typing data can be used within the registry to improve the quality and resolution of volunteer donor HLA assignments.

### **Aim B.2.1: Collection of Primary Data**

No funding was requested under this Aim for the 0339 budget cycle.

### **Aim B.2.2: Validation of Logic of Primary Data**

No funding was requested under this Aim for the 0339 budget cycle.

### **Aim B.2.3: Reinterpretation of Primary Data**

No funding was requested under this Aim for the 0339 budget cycle.

### **Aim B.2.4: Genotype Lists & Matching Algorithm**

## **Next Generation Sequencing (NGS)**

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

A meeting was held to initiate a Next-Generation Sequencing (NGS) Data Consortium in Puerto Rico at the annual ASHI meeting. This meeting brought together the main NGS technology vendors and large volume HLA typing laboratories to discuss data standards. The group identified the core elements of a Minimum Information for Reporting an Immunogenomic Genotyping Experiment (MIRIGE) MIBBI-type standard (Minimum Information for Biological and Biomedical Investigations). The presentations and outcomes of these meetings are available online at [www.immunogenomics.org/ngs](http://www.immunogenomics.org/ngs).

### **Health Level 7 (HL7)**

The NMDP became a member of HL7 (Health Level 7), and began evaluating HL7 messaging type (v3 vs v2), methodology, and development tools caBIG Life Sciences Domain Analysis Model (LS-DAM). Contact was initiated with the LS-DAM group and a plan was established to include HLA in LS-DAM (either map to current model or expand to include new concepts as needed)

NMDP staff joined the Clinical Genomics (CG) Workgroup to develop constrained CDA for reporting HLA typing. Developed 1st draft using the Model-Driven Health Tools (MDHT) from Open Health Tools. [://www.projects.openhealthtools.org/sf/projects/mdht/](http://www.projects.openhealthtools.org/sf/projects/mdht/)). A first draft of an [CDA Implementation Guide for reporting HLA](#) was created using the MDHT tool. We used the existing CDA project files which have already been constrained to create a Genetic Test Report by the HL7 Clinical Genomics Workgroup. This was further constrained using the MDHT tool. This tool allows us to automatically generate an Implementation Guide after constraining the structured document for HLA reporting requirement (Silver Standard). Required sections that were previously optional include Genetic Variation (Findings and Interpretations) and Test Information Section, which includes Background, Methodology, and References. Most of the effort to date has been on the structured data of the Test Details section and in how results can be encoded in GL String format.

- A collaboration was established with Amnon Shabo (co-chair of HL7 Clinical Genomics Work Group) to work on:
  1. Exploring use of HL7 Clinical Genomics Work Group's Genetic Testing Report (GTR) as a template for HLA typing reports. The GTR is an implementation of the HL7 CDA (Clinical Document Architecture).
  2. Developing a RMIM (Refined Message Information Model) based message that would encapsulate HML into the message
  3. Establishing collaborations with Hadassah Hospital and Northwestern University for potential pilot of exchanging HLA data in HL7 message. Hadassah and Northwestern have been communicating with the HL7 CG working group about messaging standards for tissue typing. A use case is being developed between both organizations` and NMDP to be used to develop HL7 messaging standards that embody Silver Standard principles.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- The HL7 and LSDAM resources/activities were presented to the HIEDFS group (HLA Information Exchange Data Format Standards). This is a consortium of 10 commercial HLA typing kit vendors.
- Initiated discussions with Mike Feolo and NCBI staff to develop use of GTR (Genetic Testing Registry) for meeting Silver Standard principles for methodology reporting of HLA typing. The GTR is a Clinical Document Architecture (CDA) document constrained for genetic testing by the HL7 CG working group.

### **GL String and GL Service**

Web services were developed that create, update, and retrieve HLA typing data in standardized formats without the need for allele codes and their inherent introduction of new ambiguities. ReSTful (Representational State Transfer) web services with content-aware negotiation are being developed employing a Java library that manages HLA typing data using standardized formats. These formats include the XML based Histoimmunogenetics Markup Language (HML), and a simple character delimited string format able to encode HLA typing with its ambiguity (GLString). Resources are identified with a simple URI (Universal Resource Identifier). The services access a database containing IMGT/HLA data which is updated quarterly, and objects such as alleles, lists of alleles, haplotypes, genotypes, and lists of genotypes. Public services include creating, deleting, updating, and retrieving these objects. Content negotiation allows retrieving these data in a variety of formats including GL String, HML, HTML, JSON, and QRcodes. The tools being developed provide the HLA lab a common resource for managing HLA data in a standardized way.

- A code repository was established ([://code.google.com/p/genotype-](http://code.google.com/p/genotype-)) and source code and documentation released under GNU LGPL.
- A demo was deployed of Silver Standard genotype list RESTful web service on Amazon EC2 cloud at [://gl.immunogenomics](http://gl.immunogenomics), as a foundation for feedback and presentation at the American Society for Histocompatibility and Immunogenetics (ASHI) 2012 Meeting, “Tools for Implementation of Silver Standard Principles for HLA Typing”

### **TIDES**

- A Toolkit for Immunogenomic Data Exchange and Storage (TIDES) has been developed as a demonstration of tools that use the GL Service. The tool has the ability to convert HLA genotyping data generated by three different commercial typing platforms (StripScan, HLA Fusion, and Assign ATF) into GL Strings, registering them with a GL Service, and returning URIs which are stored in a database. Further development includes: data queries, CGI upload, and format transformation, deployed on an EC2 instance on the Amazon cloud ([://tides.immunogenomics](http://tides.immunogenomics)).

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **IMMPUTE**

An initial project meeting was held with collaborators on the IMMPUTE project (to evaluate 5 algorithms performance on the imputation of HLA from SNPs in the MHC region). A detailed experimental plan has been drafted. This project includes statistical evaluation of population differentiation within the 1000 Genomes and Human Genome Diversity Panel (HGDP) datasets for the purpose of training and testing the various algorithms.

The following research groups are participating in this project:

- Bruce Weir, Department of Biostatistics, University of Washington
- Jerek Meller, Cincinnati Children's Hospital Medical Center
- Gil Mc Vean, Statistical Genetics at the University of Oxford, UK
- Paul de Bakker, Brigham and Women's Hospital and Harvard Medical School, USA
- Lue Ping Zhao, Shuying Li, Division of Public Health Sciences, Fred Hutchinson Cancer Research Center

Correlations between SNP datasets, regional (ancestry) groups and HLA types have been established and a website for distribution of datasets to participants has been established.

## **Traxis**

- A prototype implementation of Traxis was developed with changes to user-interface to display most likely alleles and show alternative genotypes. This is a pre-requisite to relaxing the need to encode HLA typing data into allele codes at the laboratory and allowing genotype lists to be reported and used for matching and on search reports.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.B. Rapid Identification of Matched Donors – Hypothesis 3:**

Registry data on HLA allele and haplotype frequencies and on the nuances of HLA typing can be used to design computer algorithms to predict the best matched donor.

### **Aim B.3.1: Phase I of Expectation Maximization (EM) Haplotype Logic**

During the grant period a new version of the algorithm was implemented (Dec 19, 2011) which includes a number of medical and technological enhancements.

The primary new functionality is that the algorithm can compute the probability that the donor typing will match the recipient at high-resolution across 5 HLA loci (HLA-A, B, C, DRB1 and DQB1) rather than the 3 loci (HLA-A, B, DRB1) used in the prior implementation. This aligns with clinical practice of considering 4 or 5 HLA loci by providing x of 8 or x of 10 match likelihood predictions. The population data resources have been greatly expanded to include haplotype frequencies derived from 5 broad and 18 detailed race/ethnic groups.

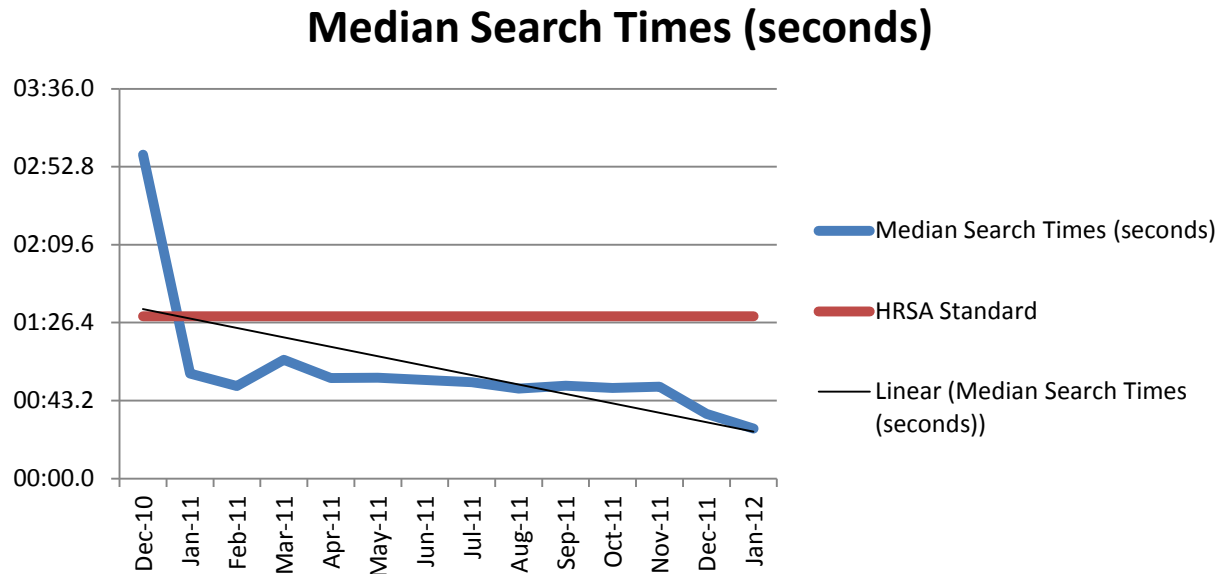
The algorithm application was migrated from the “C” programming language to Java in order to improve performance, flexibility and connectivity. The database backend was migrated from the Sybase STAR (Search Tracking and Registry) to a new Oracle database. The technical enhancements lead to a significant decrease in median search times to 35 seconds (fig. 12)

Other activity under this aim during the grant period:

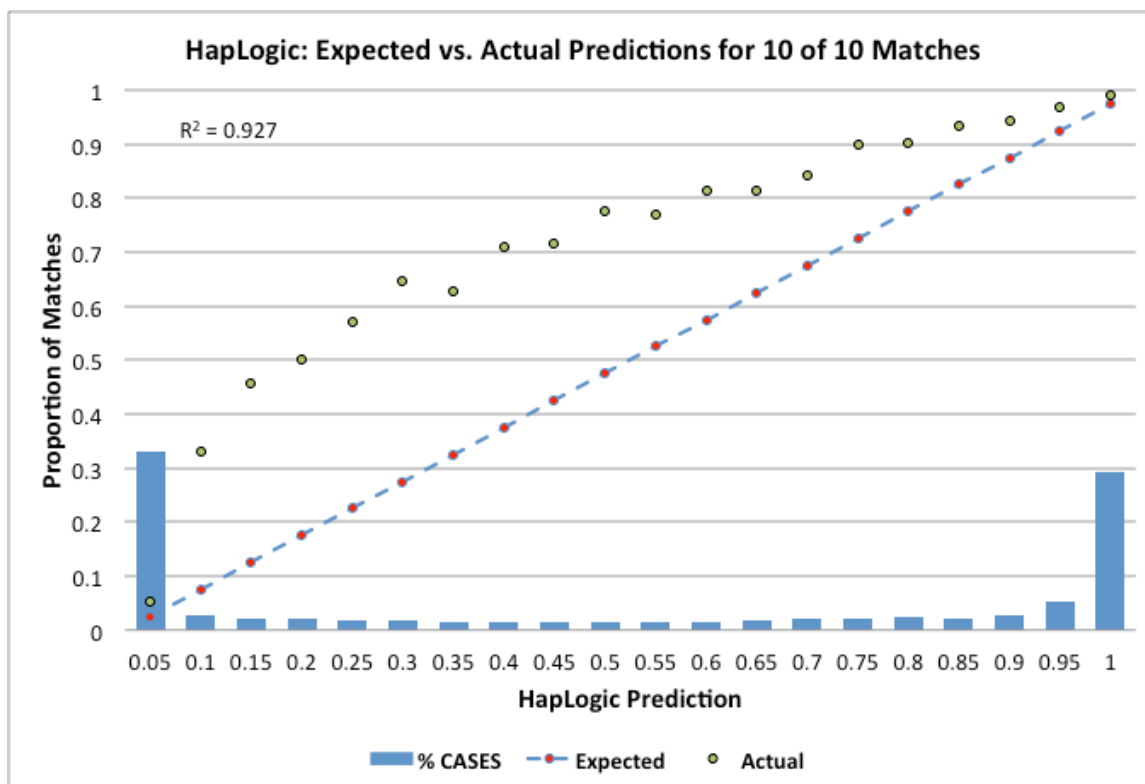
- A new webservice was created called ComputeMatchService that computes match results given any two sets of search typings. This allows access to the algorithm from other applications and for R&D use.
- Further development took place of an automated process to refresh the haplotype frequencies used for matching.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 12. Search run times over the past year.**



**Figure 13. Expected vs. actual predictions for 10 of 10 matching using the updated version of HapLogic.**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Figure 13 shows the results of a validation experiment on ~60,000 typing requests where the prediction is based on the assessment of the patient/donor combination before additional HLA typing (5%-ile categories on the X-axis) and the proportion of these cases resulted in an “actual” match (at 10 of 10 alleles) indicated with green dots on the Y-axis. The blue bars show the proportion of cases in each prediction category with the majority being at the extremes (<0.05 or >0.95). The red-dots and dashed line represents the expected proportion of matching cases. Deviation (uniformly in the direction of pessimism on the part of the algorithm) is attributable to a number of factors (insufficient haplotype frequencies, population structure, admixture or any other deviation from Hardy-Weinberg proportions) and is an area for further research.

### **Aim B.3.2 Enhancement of EM Algorithm**

#### **New Registry Haplotype Frequencies**

- New 6-locus A~C~B~DRB3/4/5~DRB1~DQB1 haplotype frequencies were computed on 21 detailed race populations and 5 rollup race populations using Blocks/Imputation EM approach on the entire DNA-typed registry.
- A testing framework was developed to evaluate the new 6-locus haplotype frequencies in a version of the HapLogic III matching algorithm and found significant improvements in predictions of allele matching compared to the production previous haplotype frequency dataset.
- A complete reinterpretation all available registry SSO probe data to HLADB 3.8.0 was completed and work was initiated to streamline the process for calculation of updated US haplotype frequencies to prepare for this to become an ongoing activity.
- The first draft of manuscript describing 6-locus haplotype frequency data was circulated to co-authors.

#### **HLA-DP Haplotype Frequencies**

- DPA1~DPB1 haplotype frequency manuscript was published in the journal Immunogenetics.<sup>2</sup>

#### **Global Haplotype Frequencies**

- A project was initiated to analyze the haplotype frequencies of all registries in BMDW. This frequency data is a pre-requisite to being able to accurately assign matching likelihoods for international donors and cord blood units presented on NMDP search reports.
- A novel method has been developed to improve 5-locus BMDW haplotype frequencies by using linkage information from similar populations. This is important to be able to provide 4 and 5-locus assignments for registry where there only 3-locus typing data.
- The IHIW registry diversity working group has the goal to validate bioinformatics methods and tools for HLA haplotype frequency analysis specifically addressing unique issues of hematopoietic stem cell registry data sets. This group met at the IHI conference in Liverpool (July 2012). A manuscript was drafted to summarize the results of the first

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

3 phases of experiments from this project.<sup>3</sup> A meeting report summarizing tasks 3, 4 & 5 for the IHIW registry diversity working group was drafted and submitted to the International Journal of Immunogenetics.<sup>4</sup>

### **Aim B.3.3 Optimal Registry Size Analysis**

An abstract on cord inventory modeling for study on CCR5-delta32 cord transplantation as potential HIV cure was accepted for an oral presentation at the 2012 BMT Tandem meetings:

- Petz, Tonai, Redei, et al. Cord blood transplantation for long term management or possible cure of HIV infection. Biol Blood Marrow Transplant 2012. 18(2s): S215. Abstract O35.

### **Aim B.3.4: Target Under-Represented Phenotypes**

#### **HaploStats**

A new version of the HaploStats application ([haplostats.org](http://haplostats.org)) was released. This is a public web-based tool to look-up haplotype frequencies based on an user-input HLA genotype. During this grant period the program was re-designed to accommodate the new haplotype frequency data from 21 detailed and 5 broad race/ethnic populations.

A series of global haplotype frequency maps have been generated and are available via the haplostats application based on links associated with the individual HLA haplotypes. This tool was presented at the 2011 EFI, ASHI and ASHG meetings. The program was also presented by Vicky Turner from St. Jude Children's Research Hospital at the 2011 IHIW conference. Additional user documentation has been added to the HaploStats application describing the new functionality.

#### **Imputation Database (IMP\_RES)**

A new relational database was developed to store the results of HLA imputation for all registry donors, patients and cord blood units. This database uses the haplotype frequencies and computational principles of HapLogic to associate a series of phased haplotype pairs with each individual HLA type. This is a foundational tool for future research initiatives and will support geospatial analysis, population modeling, CIBMTR research matching and disease association analyses.

#### **AQP**

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

An Ancestry questionnaire pilot (AQP) study was initiated to introduce enhancements to the ancestry questionnaire used by donors to join the registry. This study has been approved by the NMDP IRB and opened accrual in Oct. 2012 with the goal of enrolling 3000 subjects. This study will evaluate a novel self-identified race and ethnicity (SIRE) questionnaire and genetic ancestry informative markers in a sample of individuals from within the registry.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.B. Rapid Identification of Matched Donors – Hypothesis 4:**

Reducing the time and effort required to identify closely matched donors for patients in urgent need of HSC transplants will improve access to transplantation and patient survival in the context of a contingency response and routine patient care.

### **Aim B.4.1: Expand Network Communications**

No funding was requested for this Aim during the 0339 budget cycle.

### **Aim B.4.2: Central Contingency Management**

#### **7/8 Donor Match Rate Study**

A previous Navy funded research project estimated the 8/8 (HLA-A, B, C and DRB1) high resolution donor match rate for the four largest NMDP patient populations: Caucasian (CAU), African American (AFA), Hispanic (HIS), and Asian-Pacific Islander (API).<sup>5</sup> As a second phase, a research project was developed and completed this period to estimate the 7/8 HLA high resolution donor match rate, given this is a suitable match level for transplantation of an unrelated donor. The project also re-evaluated the 8/8 high resolution match rate which was previously determined using a 2009 donor registry snapshot. The same cohort of ‘pseudo-patient’ searches used in the 2009 study was used again to determine 7/8 match rate and to determine any updates in the 8/8 match rate since 2009. This study used a NMDP registry file from January 2012. The goal of the study was to validate previous registry benchmark analyses performed computationally and supply valuable information regarding donor selection in the event of a contingency. The project was presented to the NMDP Histocompatibility Advisory Group and approved as designed.

During the project period:

- Scientific Services experts in HLA search strategy completed donor selections on behalf of the study ‘pseudo-patients’ for four racial cohorts.
- A contract was established with an HLA testing lab.
- Donors with repository samples were tested to identify the 7/8 and/or 8/8 HLA high resolution match rate for ‘pseudo-patients’ in the four major race categories- CAU, AFA, HIS, and API.
- A total of 5130 donors and 6386 loci were typed during this project period.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- Analysis of the donor typing results was performed to determine the match rate for ‘pseudo-patients’ in the four cohorts
- 9/10 and 10/10 match rates (includes HLA-DQB1) were also determined for all four race groups

Results (nearly complete) for the project period are shown below.

Figure 14 shows the 7/8 match rate of cases by race group. A distinct difference in the 7/8 match levels between CAU (~98%) and non-CAU patients (~81-87%) is apparent.

**Figure 14. 2012 7/8 High resolution match rate.**

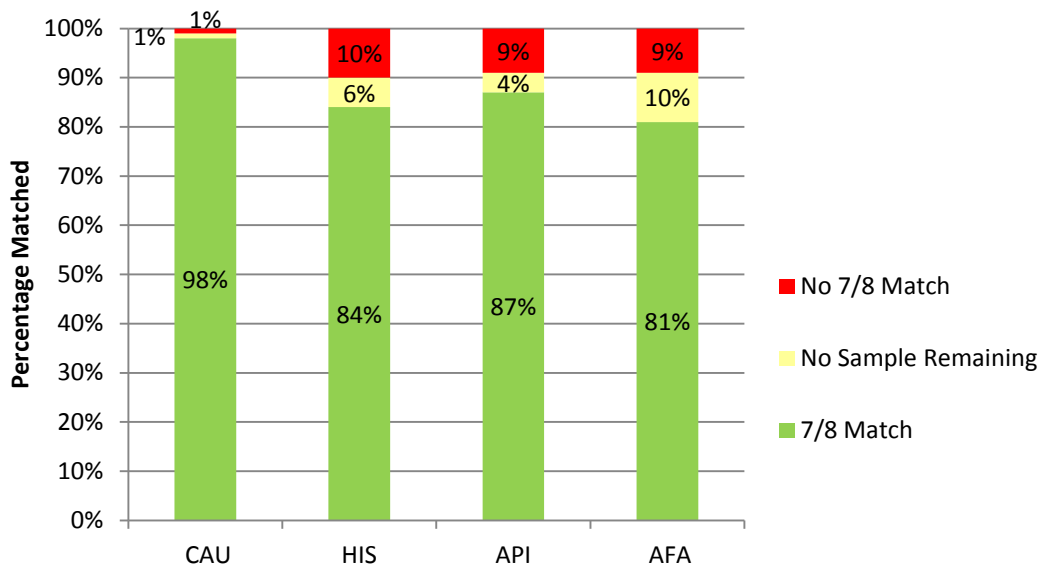


Figure 15 shows the updated 8/8 match rate of cases by race group. This graph shows the match rate differences across race groups, with CAU having the highest 8/8 matching and AFA having the lowest. These data also showed improvement from the 2009 dataset match rate of ~2-3% for each group due to an additional 3 years of donor recruitment to the NMDP registry.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Figure 15. 2012 8/8 High resolution match rate**

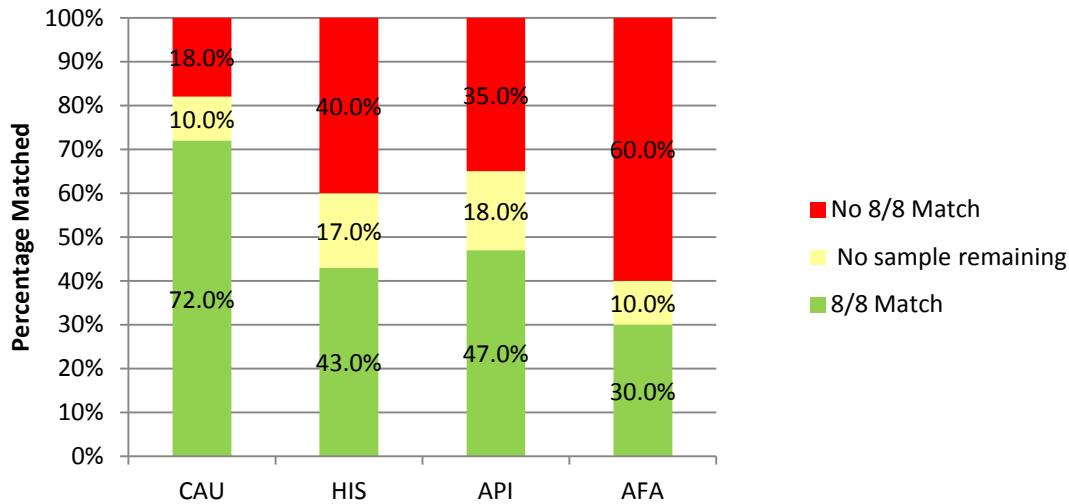
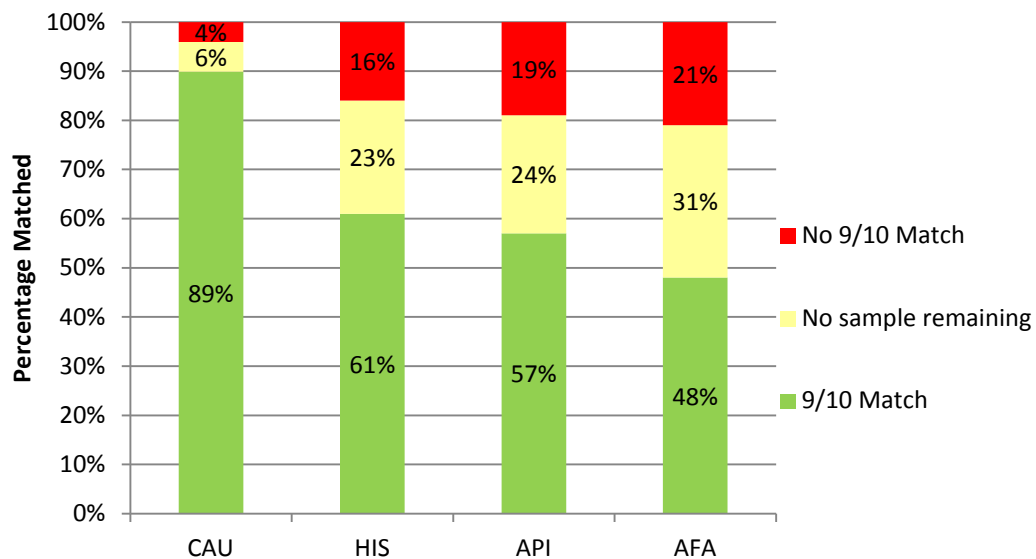


Figure 16 shows the 9/10 match rate of cases by race group. This shows the drop in match rate when HLA-DQB1 is considered. Due to tight HLA DRB1/DQB1 haplotypes, many DRB1 mismatches also result in an additional DQB1 mismatch.

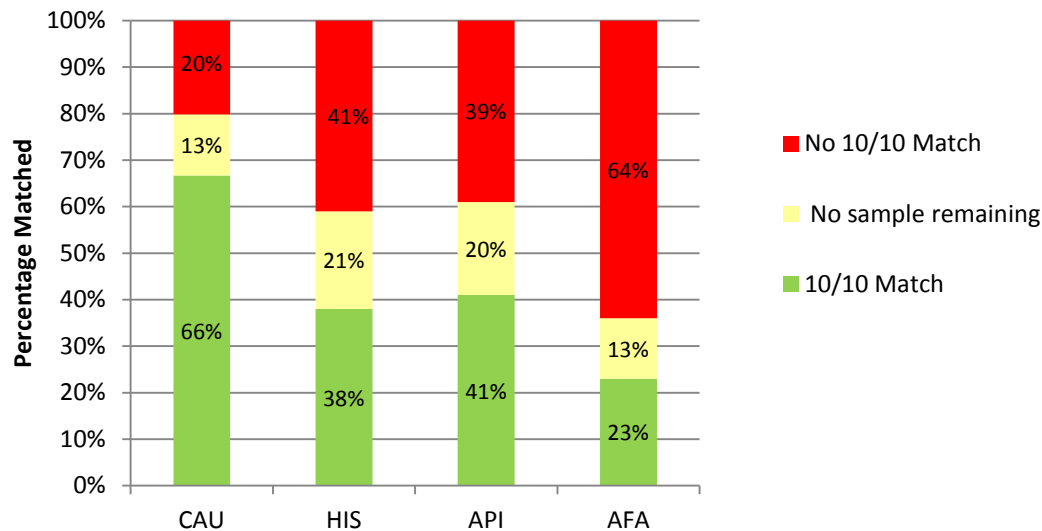
**Figure 16. 2012 9/10 High resolution match rate**



**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Figure 17 shows the updated 10/10 match rate of cases, by race.

**Figure 17. 2012 10/10 High resolution match rate**



This study is near completion. Further data analysis is being performed with plans for an upcoming abstract and manuscript. The results of this study are important for transplant center physicians and coordinators understanding of the likelihood of finding a match on the NMDP Registry when consulting with patients. This information is also vital to the NMDP's ability to understand and continue to focus on improving the match rates for incoming patients of all backgrounds. Further investigation is needed to understand the importance of retaining available donors as the match rates from this analysis are based on genetic availability. In practice, the ability of a matched donor to proceed to donation can also impact the HLA match level of a transplanted patient, with patients of non-caucasian backgrounds more likely to have only a single genetic match, an unavailable donor can mean the difference between an 8/8 and a 7/8 matched transplant.

### **Transplant Center Search Proficiency Study**

The TC Search Proficiency Study was initiated to understand the current donor selection practices of United States (US) transplant centers. The searches at each transplant center were evaluated and rated based on specific criteria to calculate a proficiency score. The results were analyzed to determine if there were transplant center factors that correlated with high search strategy proficiency.

An earlier study in 2005 occurred prior to the addition of Haplogic™ predictions and donor sorting on patient searches.<sup>6</sup> Since the 2005 study, NMDP search strategist staff have provided comprehensive search strategy training and education tracks at our annual NMDP Council

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Meetings along with webinars and presentations to internal and external staff. The two studies were compared to determine if there were changes in proficiency over time.

The 2011 TC Search Proficiency study design was reviewed and approved by the NMDP Histocompatibility Advisory Group in March 2011. The study selected and evaluated up to five random searches from each US Transplant Center. Each search was reviewed at the point of initial TC donor selection. The searches were reviewed for search proficiency which was calculated based on an algorithm to rate searches for three factors. The possible ratings (0-3 scale) for each of the three factors were summed for an overall score for each search where 9.0 was the highest rating. The three factors included:

1. Resolution of Patient HLA typing (loci and HLA resolution)
2. Adequate number of donors selected
3. Search Strategy

The searches were archived at the time of first donor selection. The study began accrual in June and continued through September 2011 when there were 556 searches evaluated from 130 TCs. The searches were stratified as high proficiency if they were rated a perfect score (9.0) or rated low proficiency if they had less than a perfect score (< 9).

- 76% rated in the high proficiency group
- 24% rated in the low proficiency group

The most frequent characteristic of searches in the low proficiency group was the selection of an inadequate number of donors. This has potential to delay a search because of the need to select, contact, and HLA type additional donors.

The results were compared to the searches evaluated in the 2005 TC Proficiency Study. The transplant centers improved their search proficiency from 52% in the high proficiency group in 2005 to 76% in 2011. From 2005 to 2011, NMDP initiated a new Custom Search Support (CSS) search service, HapLogic matching predictions and sorting, and educational initiatives which may have had an impact on this increase in searches in high proficiency group.

The study included univariate and multivariate statistical analyses of search specific factors that correlated to higher proficiency ratings. The factors and the statistical significance (using P value of <0.05) results are included below and in Table 5:

1. TC Procurement level (not significant)
2. Use of Custom Search Support (significant)
3. Use of Search Strategy Advice (not significant)
4. Presence of Certified Hematopoietic Transplant Coordinator (CHTC) on staff (significant)
5. Not a Difficult search (significant)

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Table 5. Logistic Regression Results for Search Proficiency Rating**

| <b>Factor</b>                | <b>N</b> | <b>OR</b> | <b>(95% CI)</b> | <b>p-value</b> | <b>Favorable characteristic</b> |
|------------------------------|----------|-----------|-----------------|----------------|---------------------------------|
| Procurement Level            |          |           |                 | 0.073          | Not significant                 |
| Low                          | 81       | 1.00      |                 |                |                                 |
| Medium                       | 350      | 1.89      | (0.84-4.25)     | 0.127          |                                 |
| High                         | 135      | 1.05      | (0.45-2.46)     | 0.907          |                                 |
| CSS sent to TC before Formal |          |           |                 |                | CSS sent to TC before Formal    |
| Yes                          | 71       | 4.13      | (1.39-12.28)    | 0.011          |                                 |
| No                           | 495      | 1.00      |                 |                |                                 |
| SSA sent to TC before Formal |          |           |                 |                | Not significant                 |
| Yes                          | 32       | 1.13      | (0.47-2.74)     | 0.781          |                                 |
| No                           | 534      | 1.00      |                 |                |                                 |
| At least 1 CHTC staff        |          |           |                 |                | Had at least 1 CHTC staff       |
| Yes                          | 239      | 2.07      | (1.22-3.50)     | 0.007          |                                 |
| No                           | 327      | 1.00      |                 |                |                                 |
| Difficult search             |          |           |                 |                |                                 |
| Yes                          | 226      | 0.63      | (0.42-0.93)     | 0.020          | Not difficult                   |
| No                           | 340      | 1.00      |                 |                |                                 |

OR= odds ratio; CI= confidence interval

The TC Search Proficiency Study initial results (accrual through September 2011) were presented as a poster abstract at the February 2012 BMT Tandem annual meeting.<sup>7</sup>

Study enrollment continued through December 2011 with the addition of 53 searches and two transplant centers. The final data set consists of 619 searches from 132 transplant centers. The final data are being used for statistical analysis and the results will be prepared as a manuscript planned for early 2013.

### **Additional Presentations**

Kevin Tram, et al, Considering HLA-C matching for single cord blood unit transplants, Poster presentation at 2012 ASHI annual meeting. In brief, a cohort of primarily pediatric single CBU transplants from December 2011 to February 2012 (n = 119) was examined to determine if a more fully matched x/8 (antigen level at HLA-A, B, C and allele level at HLA-DRB1) unit was available in the Be The Match Registry®. Of the 119 single CBU transplants assessed, 79 (66%) of the transplants had the best matched unit while 40 (34%) had a better matched unit available. The cell dose of the better matched unit was higher in 42.5% of cases, with the median change in cell dose of -9.3%.<sup>8</sup>

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Jason Dehn, et al, A/B only typed donors: Possible but improbable 6/6 HLA allele matches, Poster presentation at 2012 ASBMT/CIBMTR Annual Tandem Meeting. In brief, we identified 180 new patient searches with no potential 6/6 HLA allele matched donors, reviewed the search results, and typed DRB1 on a total of 2,976 A/B only donors found on the patient searches with stored DNA samples in attempt to identify a matched donor. Of those tested, two donors matched their respective patient at 6/6 and one donor became the only 10/10 allele matched donor. That patient proceeded to a successful transplant. Overall, this study showed limited benefit to testing donors who are only typed at HLA-A/B, so strategies to pursue this testing may be best accomplished in tandem with the selection of the best mismatched donors. Future studies evaluating how the A/B only pool can support finding the best 9/10 or 7/8 match for patients are being considered<sup>9</sup>

**Aim B.4.3 Conduct a transplant center benchmarking analysis to identify center-specific factors (e.g., quality management techniques and processes) that contribute meaningfully to superior survival outcomes. Share processes that contribute to superior outcomes with the entire TC network as best practices.**

No funding was requested under this Aim for the 0339 budget cycle.

**Aim B.4.4 Identify plans to expand capabilities of collection center and apheresis center network to meet increasing number of donor product requests on both a short-term and long-term basis.**

No funding was requested under this Aim for the 0339 budget cycle.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.C. Immunogenetic Studies – Hypothesis 1:**

HLA mismatches may differ in their impact on transplant outcome, therefore, it is important to identify and quantify the influence of specific HLA mismatches. In contingency situations it will not be possible to delay transplant until a perfectly matched donor can be found.

### **Aim C.1.1: Donor Recipient Pair Project**

In 1994 a retrospective D/R Pair HLA typing project to characterize class I and class II alleles of donor/recipient paired samples from NMDP's Repository was initiated. The goals of this ongoing research project are to assay the impact of DNA-based HLA matching on unrelated donor transplant outcome, develop strategies for optimal HLA matching, evaluate the impact of matching at alternative HLA loci on transplant outcome and finally to promote the development of DNA-based high resolution HLA typing methodologies. Presence/absence typing of 14 KIR loci (2DL1-5, 2DS1-5, 3DL1-3 and 3DS1) has been included.

- Typing was completed and final results were received on the 295 umbilical cord blood/recipient pairs of 110 unrelated donor/recipient pairs. All 405 pairs were typed for HLA and KIR. Linkage, no make and discrepancy resolution was initiated.
- To date over 2400 pairs and 1180 additional donors have been typed for presence/absence of 14 KIR loci (2DL1-5, 2DS1-5, 3DL1-3 and 3DS1).

Current HLA matching guidelines for unrelated HCT recommend avoidance of mismatches only within the Antigen Binding Domain (ABD). This recommendation is based on the hypothesis that amino acid differences outside the ABD are not immunogenic. The ABD allo-reactivity assessment project will give insight into the allowable percent tolerance of matching needed outside of the ABD.

We initiated investigation of a class II non-ABD mismatch (DRB1\*14:01:01/14:54) where both alleles were seen in the same genotype. Seventy two donors were invited to participate in the study. Twenty one study participants consented and submitted blood samples. Eleven donor pairs representing four distinct haplotypes pairs were selected for in vitro testing. The initial results showed 1 of 6 tested combinations to be alloreactive to the non-ABD mismatch in both the mixed lymphocyte culture and ELISPOT assays. Confirmatory tests were performed to determine whether the alloreactivity was due to the non-ABD mismatch or disparity at HLA-

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

DRB3, which was mismatched in the specimens. The confirmatory tests will incorporate blocking antibodies to DRB3 to isolate the impact of the DRB1 disparity.

Initial investigation of the class I non-ABD mismatches (A\*02:01/02:09, B\*44:02/44:27 and C\*07:01/07:06) where both alleles are believed to be in the same genotype have been performed. Specific queries of the Be The Match Registry allowed for selection of one hundred and forty potential donors to be typed at high resolution for the class I locus of interest. Class I typing was performed and it was determined that the B\*44:02/44:27 would be the best candidate for complete typing. Typing at all loci for 53 donors occurred but results showed that the alleles did in fact segregate to two distinct haplotypes and are not useful for further testing. Analysis of other potential alleles is ongoing.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.C. Immunogenetic Studies – Hypothesis 2:**

Even when patient and donor are HLA matched, GVHD occurs so other loci may play a role.

### **Aim C.2.1: Analysis of non-HLA loci**

Recent research has heightened interest in additional genetic polymorphisms which may modify the outcomes of transplantation. HLA genes other than the major histocompatibility complex (MHC) found on chromosome 6 and non-HLA genetic factors may all influence the suitability and success of allogeneic stem cell transplants.

#### **Immunobiology Integration DataBase (IIDB)**

This database was developed to allow the integration of research HLA typing and repository sample data from NMDP with the clinical outcomes data from CIBMTR. A monthly process to generate an “HLA Save file” with HLA and match grades for clinical outcomes research has been integrated into the IIDB database after significant progress in quality assurance testing. A “data quality” schema was created for collection of HLA validation error to be used for metrics and error resolution when validating HLA data from external sources and CIBMTR outcomes forms.

#### **Clinical Ancestry Study**

A new study proposal has been submitted to the CIBMTR Immunobiology Working Group to study the effect of matched genetic ancestry of donors and patients on transplant outcomes. This study was accepted and preliminary experiments have been run to investigate SNP panels as analysis tools for this study. A poster describing this study was presented at the 2012 BMT Tandem meetings.

#### **The Immunobiology Project Results (IPR) database**

This database and its applications allow for storage and analysis of all immunogenetic data collected on NMDP research samples. This database has replaced the existing HLA donor/recipient pair’s database and facilitates storage and analysis of data from other immunogenetic loci.

During this period many bug fixes and minor enhancements within both reports and tools have been performed. Full support for multi-donor transplants has been added and several enhancements to improve the look-and-feel of the application were completed.

Key accomplishments during the last period:

1. Hover-over expansion of allele codes in reports and tools
2. Expanded capability to search for alleles. The entire database may now be searched
3. Migrated historical data from the High Res database into IPR

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

4. Migrated the IPR applications to more powerful servers
5. New reports produced
  - a. ID
  - b. Completed Pairs
  - c. Discrepancy
  - d. N of X
  - e. Pre and post DRB1, 3/4/5 and pre and post B/C linkage
  - f. Study data representation

Immunobiological test results generated through NMDP/CIBMTR approved studies and reported to the NMDP are summarized in Table 6. These data will be used for testing, validation, and population of the IPR database.

**Table 6. Immunobiology typing projects utilizing NMDP samples and contributing data to the IPR database**

| Study Title                                                                                                                                          | Investigator      | Number of Samples | Genes of interest              | Testing Method               | Data Submitted |
|------------------------------------------------------------------------------------------------------------------------------------------------------|-------------------|-------------------|--------------------------------|------------------------------|----------------|
| NK Cells, Their Receptors and Unrelated Donor <sup>,11</sup>                                                                                         | J. Miller         | 2300 pairs        | KIR                            | RT-PCR, FACS, SSO, MALDI-TOF | Yes            |
| Survey of Diversity of Immune Response Genes in Unrelated Hematopoietic Stem Cell Transplantation                                                    | C. Hurley         | 40 Pairs          | cytokine and KIR               | SBT                          | Yes            |
| Candidate Gene Study to Examine the Impact of Chemokine and Chemokine Receptor Gene Polymorphisms on the Incidence and Severity of Acute and Chronic | R. Abdi           | 1300 pairs        | CCL1, CCL2, CCR5, CCR2, CX3CR1 | Taqman PCR                   | Yes            |
| Functional Significance of Killer Ig-like Receptor (KIR) Genes in HLA Matched and Mismatched Unrelated                                               | B. Dupont, K. Hsu | 2000 pairs        | KIR                            | SSP                          | Yes            |
| Functional Significance of Cytokine Gene Polymorphism in Modulation Risk of Post-Transplant Complications                                            | E. Petersdorf     | 2500 pairs        | >30 Immune response genes      | Taqman PCR                   | Yes            |
| Identification of Functional SNPs in Unrelated <sup>,16</sup>                                                                                        | E. Petersdorf     | 3500 pairs        | Entire MHC region              | Taqman PCR                   | In Process     |

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

| <b>Study Title</b>                                                                                                                                             | <b>Investigator</b> | <b>Number of Samples</b> | <b>Genes of interest</b>                     | <b>Testing Method</b>            | <b>Data Submitted</b> |
|----------------------------------------------------------------------------------------------------------------------------------------------------------------|---------------------|--------------------------|----------------------------------------------|----------------------------------|-----------------------|
| Use of Female Donors with Pre-existing Antibody to H-Y Antigen will Result in Robust Serologic Response to H-Y Antigens in Male HSC transplantation Recipients | D. Miklos           | 288 pairs                | H-Y Antigen                                  | ELISA, protein array             | Yes                   |
| Multiplexed Genotyping of Human Minor Histocompatibility Antigens (mHAg): Clinical Relevance of mHAg Disparity in Stem Cell                                    | T. Ellis            | 730 pairs                | mHAg                                         | Allele-specific Primer Extension | Yes                   |
| Genetic Polymorphisms in the Genes Encoding Human Interleukin-7 Receptor- $\alpha$ : Prognostic significance in Allogeneic Stem Cell                           | K. Muller           | 851 pairs                | IL-7                                         | Taqman PCR                       | Yes                   |
| The Effect of Non-Inherited Maternal Antigens in Cord Blood                                                                                                    | L. Baxter-Lowe      | 102 pairs                | HLA                                          | SBT                              | Yes                   |
| Detection of HLA Antibody in Single Antigen HLA-Mismatched Unrelated Donor Transplants                                                                         | S. Arai, D. Miklos  | 200 pairs                | Anti-body                                    | ELISA, Protein array             | Yes                   |
| Detection of Donor-Directed, HLA-Specific Alloantibodies in Recipients of Unrelated Stem Cell Transplantation and Their Relationship to Graft/Patient          | R. Bray             | 111 pairs                | Anti-bodies                                  | Flow cytometry                   | Yes                   |
| Genome-wide Association in Unrelated Donor Transplant Recipients and Donors: A Pilot Study                                                                     | R. Goyal            | 858 pairs                | > 600,000 Genome wide SNPs                   | Human 610 - Quad V1 arrays       | In process            |
| SNPs in the p53 Pathway and Outcomes in URD HCT                                                                                                                | B. DuPont           | 1500 pairs               | p53, ATM, MDM2 and p21/Waf1                  | Taqman                           | In process            |
| Association of Donor and Recipient Gene Polymorphisms of Drug and Innate Immune Response with Outcomes after URD HCT                                           | V. Rocha            | 725 pairs                | GSTP, GSTT, GSTM, UGT CD14, TIRAP, and NALPs | Taqman                           | In process            |

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

| <b>Study Title</b>                                                                                                                                      | <b>Investigator</b>                               | <b>Number of Samples</b> | <b>Genes of interest</b>                       | <b>Testing Method</b>                   | <b>Data Submitted</b> |
|---------------------------------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------|--------------------------|------------------------------------------------|-----------------------------------------|-----------------------|
| To Develop and Test a Prognostic Index for Survival in CML URD                                                                                          | A. Dickinson                                      | 1100 pairs               | TNF, IL-1RA and IL-10                          | Taqman                                  | Yes                   |
| Evaluation of TGF- $\beta$ 1 Promoter and Signal Peptide Polymorphisms as Risk Factors for Renal Dysfunction in HCT Patients Treated with Cyclosporine  | R. Shah                                           | 400 samples              | TGF- $\beta$ 1                                 | Taqman                                  | Yes                   |
| Donor and Recipient Telomere Length as Predictors of Outcomes after Hematopoietic Stem Cell Transplant in Patients with Acquired Severe Aplastic Anemia | S. Gadalla                                        | 650 samples              | Telomere length and Telomerase Polymorphisms   | Taqman                                  | In process            |
| Development of a GVHD Prevention Biodiagnostic Test                                                                                                     | R. Somogyi                                        | 450 samples              | Gene Expression Array                          | Array                                   | In process            |
| Genetic polymorphisms and HCT related mortality Re: Pre-HCT conditioning in matched unrelated donor HCT                                                 | T. Hahn                                           | >4,000 pairs             | GWAS                                           | Array                                   | In process            |
| Impact of CTLA4 SNPs on outcome after URD transplant                                                                                                    | M. Jagasia                                        | 1,200 pairs              | CTLA-4 SNPs                                    | Taqman                                  | In process            |
| KIR genotyping and immune function in MDS patients prior to unrelated donor transplantation                                                             | A. E. Warlick and J. Miller                       | 970 samples              | KIR genotype, expression and cellular function | SSP, flow cytometry and cellular assays | In process            |
| Plasma YKL-40 and CHI3LI genotype to predict mortality after unrelated donor HCT                                                                        | B. Kornblit                                       | 800 pairs                | YKL-40 plasma levels and CHI3LI SNPs           | ELISA and Taqman                        | In process            |
| Natural killer cell genomics and outcomes after allogeneic transplantation for lymphoma                                                                 | V. Bachanova, J. Miller, D. Weisdorf and L. Burns | 800 pairs                | KIR genotype, expression and cellular function | SSP, flow cytometry and cellular assays | In process            |
| Effect of genetic ancestry matching on HSCT outcomes                                                                                                    | A. Madbouly, M. Maier and N. Majhail              | 2300 pairs               | Ancestry Informative Markers                   | Taqman                                  | In process            |

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Aim C.2.2: Related Pairs Research Repository**

No funding was requested under this aim for the 0339 budget cycle.

**Aim C.2.3 CIBMTR Integration**

No funding was requested under this aim for the 0339 budget cycle.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

## **II.D. Clinical Research in Transplantation – Hypothesis 1:**

Clinical research in transplantation improves transplant outcomes and supports preparedness for a contingency response.

### **Aim D.1.1: Observational Research, Clinical Trials, and NIH Transplant Center**

#### **Resource for Clinical Investigations in Blood and Marrow Transplantation**

During this grant, the Resource for Clinical Investigations in Blood and Marrow Transplantation (RCI BMT) developed its infrastructure further and made progress on a number of studies. The goal of this program is to provide an avenue for investigators to obtain statistical, study and data management support for prospective trials focusing on addressing various transplant issues. The following key elements were accomplished:

- The Clinical Trials Advisory Committee (CTAC), whose mission is to provide scientific review and recommendations on clinical trial proposals met a total of three times during this period. Twice in person (Tandem 2011 and 2012) and one conference call meeting during July 2012. A total of five study proposals were reviewed. Two were not recommended to proceed, one was recommended but the company decided to withdraw their proposal while the remaining two required additional information and a request to resubmit.
- Managed the Adult Double Cord in patients with hematologic malignancies trial. During this grant, accrual was met with a total of 56 patients enrolling. Staff continued to coordinate study activities, manage data and perform on site monitoring. A Tandem 2013 abstract was developed, submitted and accepted for oral presentation.<sup>22</sup> Initial work also began on analysis and writing of a full manuscript.
- During this grant period, the Lenalidomide after allogeneic HCT for Myeloma trial also met its accrual goal with 30 patients. Data management activities continued and a Tandem 2013 abstract was accepted for oral presentation.<sup>23</sup> In addition, further data analysis and writing of a full manuscript has begun.
- Staff supported the continued efforts related to the BMT CTN PBSC vs Marrow Phase III trial. This included managing the donor follow up component of the study and site monitoring at the donor centers. During this grant the Survey Research Group (SRG) acquired the five-year time point of the recipient quality of life and fully integrated this effort into their activities.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- The Long-term Donor Follow-up protocol continued to accrue donors both from the previous donated group and prospectively as donors participated in the standard work-up process. At the end of this grant period close to 10,000 donors were enrolled. The Survey Research Group is responsible for completing the follow up time points for the NMDP Operated Donor Centers. During this grant period they completed 5,629 follow up forms. As part of the project there was a plan to make a last outreach to those donors in the previously donated group who had not responded to the first three outreaches for consenting to the study. At the end of this grant period, 96% of the ~7000 in this group were contacted and 24% enrolled, 4% declined and the remaining were either unreachable or never responded. The SRG also absorbed into their process for following up on any reported events. This includes requesting a release of medical records from the donors, obtaining the records and preparing them for review by the Medical review team.
- As part of the CMS-MDS study accrual and data submission occurred during this grant period. Transplant Centers with a recipient enrolled on this study are required to submit the comprehensive research forms. A system is in place to only select a limited number of forms for general research purposes due to limited funds. The addition of these forms to the selection criteria does not replace but adds to the required forms. A total of 82 recipient CRF payments were covered during this period.
- During this grant period staff from the RCI BMT worked with CIT staff to complete operational requirements for a) comprehensive system for management of activities and studies within the Survey Research Group and b) clinical trial management system (CTMS) software to coordinate operational and administrative activities within RCI BMT.

### **Cord Blood Research Activity**

During the project period, the Cord Blood Research subcommittee met semi-monthly to discuss study priorities and plan analyses for the following:

- The Duke and MD Anderson laboratory staff completed work on validating the assay methodologies but were unable to ensure consistent results generated at both testing sites for the study investigating biomarkers associated with cord blood engraftment. Initial and final statistical analysis of the validation testing results showed poor inter-laboratory reliability for all assays performed. Therefore, testing using a third laboratory was developed with St. Louis Cord Blood Bank (SLCBB) to determine whether the poor reliability is due to center-specific or assay related issues.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- The Duke and SLCBB created and finalized plans and testing for training and validating the assay methodologies. The data analysis for the results of the training phase met the acceptable threshold for the inter-laboratory reliability coefficient of variation (CV); however, the validation phase data analysis did not. An investigation into the cause of the poor reliability is on-going and does not involve further testing of samples.
- A white paper detailing recommendations/guidelines for the assessment of new assays (potency or other assays) relevant to cord blood banking and/or transplantation was published in . As a result, the NMDP was contacted by Hemogenix regarding a collaborative validation study for their potency assay (HALO). The proposal was reviewed by the Cord Blood Advisory Group (CBAG) and the Cord Research Subcommittee. The subsequent protocol was developed and reviewed and approved by the CBAG. Plans are in place to proceed with protocol development. Members of the CBAG expressed interest in the validation; however, upon solicitation of member banks for completion of the validation none could commit to the effort due to other priorities. The cord blood banks expressed a need to focus on FDA licensure efforts.
- The analysis evaluating the likelihood of finding a non-inherited maternal antigen/allele (NIMA) match for HLA mismatched cord blood unit for transplant when upfront maternal typing is not available was completed. The retrospective analysis compared the frequencies of the NIMA matched and mismatched HLA- A, B antigens or DRB1 alleles found in the Eurocord/NMDP/CIBMTR study to determine any significant differences. Results were incorporated into a manuscript which was published in Blood and Marrow Transplant Research.<sup>25</sup>
- Work began on a study to assess CBU characteristics (viability, TNC, CFU and CD34) pre-freeze and post thaw. Segment evaluation prior to unit release is under consideration as a third evaluation point, but will require an understanding of the release testing performed by the various CBBs. A survey was sent out to Network cord blood banks to collect data on cord blood release testing practices. Results of a survey to the cord blood banks were analyzed and the unit release testing data deemed too variable for meaningful analysis. The study will proceed with pre-freeze and post-thaw characteristics only. The study was discussed during the Cord Blood Advisory Group in May. A task force for study development was created and conference calls to discuss study development were conducted. The study design and proposal for submission to the CIBMTR was completed, submitted and accepted for presentation at the Graft Sources and Manipulation Working Committee at the BMT Tandem Meetings in 2013.
- Development of the anti-HLA donor specific antibody study of recipients transplanted with cord blood units was initiated. It was determined that the study cohort was too small to proceed with the study at that time.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- Work was initiated and completed on an assessment of the impact of donor inherited paternal antigen (IPA) disparity on outcomes after unrelated cord blood transplantation (UCBT) for acute lymphoblastic leukemia and acute myelogenous leukemia. Results were detailed in an abstract submitted and accepted for poster presentation at the 2012 International Cord Blood Symposium.
  - Colleen Brady, et al., Impact of donor inherited paternal antigen (IPA) disparity on outcomes after unrelated cord blood transplantation (UCBT for Acute Lymphoblastic Leukemia and Acute Myelogenous Leukemia. Poster presentation 2012 International Cord Blood Symposium.
- Two cord blood workshops were developed and conducted at the 2011 NMDP Council Meeting. Each workshop was well attended and received excellent ratings from the attendees.
  - New Research to Improve Cord Blood Transplant Outcomes: This workshop offered some of the cutting-edge work focused on improving cord blood outcomes by addressing some of the limitations of the graft source.
  - New Cord Blood Matching Concepts: This workshop provided recent findings on extended HLA matching (role of the HLA-C locus), matching for non-inherited maternal antigens, and the impact of pre-existing cord specific anti-HLA antibodies in cord blood transplant recipients.
- Two cord blood workshops were developed and conducted at the 2012 NMDP Council Meeting. Each workshop was well attended and received excellent ratings from the attendees.
  - Cord Blood Transplantation for Non-Malignant Disease: The workshop described the use of umbilical cord blood transplant (UCBT) for the treatment of nonmalignant diseases, specifically sickle cell anemia and hemoglobinopathies.
  - Unrelated Donor and CBU Selection Guidelines: This workshop presented updated selection guidelines from the NMDP and CIBMTR based on current and relevant data correlating graft characteristics with clinical transplant outcomes, on appropriated typing strategies and matching criteria for unrelated adult donor and cord blood graft selection.

*NIH Search Support*

The National Institutes of Health (NIH) has been accepted as an NMDP transplant center since 2007. Prior to that time, the NIH, representing our Nation's premier medical research endeavor, was not applying their considerable problem-solving skills to issues surrounding unrelated donor transplantation. The NMDP, with ONR support, set out to remedy that deficiency by entering into collaboration with NIH. This collaboration has been extremely successful.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

The NMDP is collaborating with intramural NIH transplant programs from the NCI, the NHLBI and the NIAID. These programs are investigating alternative approaches in unrelated donor transplantation to improve patient outcomes. The actual transplants and the investigational portions of each transplant (i.e., the research protocols) are supported entirely with NIH funds. Navy funding supplies support for donor identification, selection, and collection. NMDP donors are not research subjects on these protocols because the donors are making standard donations for accepted transplant indications. The research component of these transplants is conducted entirely by NIH intramural program staff and funded entirely with NIH dollars. The NMDP provided support for the collection of 35 products (25 PBSC, 9 CBU, and 1 therapeutic T cell) under the grant.

### **CIBMTR Observational Research**

Support of the Observational Research program included statistical hours for managing studies within the Immunobiology (see section IID1.3 below), GVHD, and Graft Sources Working Committees. During this grant period staff performed proposal review, protocol development, data preparation, data analysis, and manuscript preparations. Details regarding the Immunobiology activities can be found in IID1.3 below. The GVHD and Graft Sources Working Committees published 9 manuscripts.<sup>26-34</sup> During the grant period, staff performed various other functions on over 20 other studies.

### **CIBMTR IT Activity**

During the project period, significant strides were made in improving user experience, delivering new functionality, improving data quality, data capture and data reporting through the CIBMTR IT suite of applications.

### **Audit**

- Implementation of Donor Audit and Monitoring Functionality into FormsNet. This aids the audit team by providing the materials required to conduct on-site audits of Donor Centers (DC) to determine and document the error rate in the FormsNet2 database and identify systematic and non-systematic errors.

### **Prospective Studies**

- A Sample Tracking Application in support of Clinical Trials was implemented into production in June 2011, along with the 1st clinical trial it supports. This application provides sample management/inventory tracking functionality for clinical trials. The associated Clinical Trial that was implemented was the 09-MRD (Minimal Residual Disease) Study. The new Form 2007 (Cord Blood) was also implemented into FormsNet.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

- An additional release was completed to automate monitoring of a clinical trial. This functionality increased data quality and improved the efficiency in preparing data for analysis for Observational Studies.

### **Management Reporting Website**

- Documented, produced, and released 35 new reports based on FormsNet data. This included clinic trials support, and management oversight of the Continuous Process Improvement Phase IV where centers must meet a 90% completed form submission and have a current IRB approved consent for research data.

### **NMDP Legacy Data**

- Quality assurance of the mapping of NMDP legacy data (before 11/2007) to the FormsNet database was completed and migrated to FormsNet2.

### **Cord Blood/ Event Reporting**

- This functionality was implemented in FormsNet to support the 10-CBA (Cord Blood Access) study and Event Reporting scope. This effort delivered components for enrollment, data collection of adverse events and product deviations (7 forms), tracking and reporting and medical monitoring. This project delivers functionality to meet the FDA requirements to collect and display Licensure status and IND information on CBUs by October 2011.

### **FormsNet3**

- FormsNet3 was implemented after the funding period. However, the requirements documentation, extensive design and delivery of the technical infrastructure were a major focus of effort during the funding period.
- FormsNet3 Phase 1 – Recipient development remained on tract during this period and was undergoing full regression testing at the end of the funding period.

### **AGNIS & Metadata**

- Through this period the AGNIS and metadata teams have enhanced their collaborative relationship with NCI by sharing tools developed by the AGNIS team with NCI for use of non-CIBMTR curation teams.
- Worked with NCI to integrate current CIBMTR context for hematopoietic cell transplantation (HCT) into future data model systems, such as Biomedical Research Integrated Domain Group (BRIDG). 1500 CIBMTR data points were approved.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**EBMT**

- European Group for Blood and Marrow Transplantation (EBMT)/ Eurocord Project. CIBMTR IT facilitated the connection of EBMT and Eurocord data submission to AGNIS. This exchange of data with European registries and cord blood banks is particularly beneficial to enhance cord blood outcomes data. Currently 48 centers are participating in production submission.

**Center Activity**

- There are 3 centers submitting to AGNIS without vendor support
- There are 12 centers certified to submit to AGNIS through vendor relationships
- There are 26 centers certified for retrieving data from AGNIS through vendor relationships

**Forms supported**

- Form2000 - Recipient Baseline Data
- Form2004 - Infectious Disease Markers
- Form2005 - Confirmation of HLA Typing
- Form 2006 – HSCT Infusion FormForm2018 - Hodgkin and Non-Hodgkin Lymphoma Pre-HSCT data
- Form2100 - 100 Days Post-HSCT Follow-up Form
- Form2118 - Hodgkin and Non-Hodgkin Lymphoma Post-HSCT data
- Form2200 - Six Months to Two Years Post-HSCT Data
- Form2300 - Yearly Follow-Up for Greater Than 2 Years Post-HSCT data
- Form2400 - Pre-Transplant Essential Data
- Form2450 - Post-Transplant Essential Data
- Form2451 - Chimerism Studies
- Form2455 - Selective Post-Transplant Essential Data
- Form 2804 – Unique ID form
- Form2900 - Recipient Death Data

**Aim D.1.2: Research with NMDP Donors**

No funding was requested under this aim for the 0339 budget cycle.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Aim D.1.3: Expand Immunobiology Research**

During a previous grant period, the NMDP developed the Immunobiology Research grant request and award procedures for use by the IBWC and developed the IBWC Web site ([://www.cibmtr.org/Studies/Immunobiology/Pages/index](http://www.cibmtr.org/Studies/Immunobiology/Pages/index) ). The content was updated on a regular basis during the grant period to highlight research activities, solicit new study proposals and feature the unique research resources (CIBMTR database, biostatistical expertise and NMDP Research Repository) available through the IBWC.

Grant funds supported significant outreach efforts by the IBWC leadership to increase exposure for the IBWC to researchers involved in immunobiology and immunogenetics. The IBWC leadership had a presence at the American Society of Hematology, BMT Tandem, European Group for Blood and Marrow Transplant, European Federation of Immunogenetics, and Cord Blood Symposium annual meetings.

In addition, the IBWC co-scientific director and biostatistician participated in the 16th International Histocompatibility Workshop in May 2012. The CIBMTR IBWC plays an integral role in the IHIW HCT component by supporting data and research sample sharing for all U.S. transplant centers that participate in the CIBMTR and NMDP Research Repository. The co-scientific director also serves on the IHIW-HCT steering committee.

Support permitted the IBWC to maintain a strong performance record with 6 abstracts accepted for presentation, 9 manuscripts published, and 1 manuscript submitted for publication. The IBWC reviewed 8 new proposals during the BMT Tandem meetings in February 2012 and accepted 6 for analysis. The IBWC leadership approved an immunobiology research grant to support the costs associated with the use of NMDP Research samples for study IB12-05: Plasma YKL-40 and CHI3L1 Genotype to Predict Mortality after Allogeneic Hematopoietic Cell Transplantation.

Nine manuscripts were published:

1. Spellman SR, Eapen M, Logan BR, Müller C, Rubinstein P, Setterholm MI, Woolfrey AE, Horowitz MM, Confer DL, Hurley CK. A perspective on the selection of unrelated donors and cord blood units for transplantation. *Blood*. 2012 Jul 12; 120(2):259-265. doi:10.1182/blood-2012-03-379032. Epub 2012 May 17. PMC3398760.
2. Battiwalla M, Wang T, Carreras J, Deeg HJ, Ayas M, Bajwa RPS, George B, Gupta V, Pasquini R, Schrezenmeier H, Passweg JR, Schultz KR, Eapen M. HLA-matched sibling transplantation for severe aplastic anemia: impact of HLA DR15 antigen status on engraftment, graft-versus-host disease, and overall survival. *Biology of Blood and Marrow Transplantation: Journal of the American Society for Blood and Marrow Transplantation*. 2012 Sep 1; 18(9):1401-1406. doi:10.1016/j.bbmt.2012.02.007. Epub 2012 Feb 28. PMC3406237.

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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5. Horan J, Wang T, Haagenson M, Spellman SR, Dehn J, Eapen M, Frangoul H, Gupta V, Hale GA, Hurley CK, Marino S, Oudshoorn M, Reddy V, Shaw P, Lee SJ, Woolfrey A. Evaluation of HLA matching in unrelated hematopoietic stem cell transplantation for nonmalignant disorders. *Blood*. 2012 Oct 4; 120(14):2918-2924. doi:10.1182/blood-2012-03-417758. Epub 2012 Jul 24. PMC3466972.
6. Petersdorf EW, Malkki M, Gooley TA, Spellman SR, Haagenson M, Horowitz MH, Wang T. MHC Resident Variation Affects Risks after Unrelated Donor Hematopoietic Cell Transplantation. *Sci Transl Med*. July 25; (144): 144ra101. : 10.1126/scitranslmed.3003974.
7. Venstrom JM, Pittari G, Gooley TA, Chewning JH, Spellman SR, HLA-C–Dependent Prevention of Leukemia Relapse by Donor Activating KIR2DS1. Submitted to *New England Journal of Medicine*. *N Engl J Med*. 2012 Aug 30;367(9):805-16. doi: 10.1056/NEJMoa1200503.
8. Rocha V, Spellman S, Zhang MJ, Ruggeri A, Purtill D, Brady C, Baxter-Lowe LA, Baudoux E, Bergamaschi P, Chow R, Freed B, Koegler G, Kurtzberg J, Larghero J, Lecchi L, Nagler A, Navarette C, Prasad V, Pouthier F, Price, T, Ratanatharathorn V, van Rood JJ, Horowitz MM, Gluckman E, Eapen M. Effect of HLA-matching recipients to donor noninherited maternal antigens on outcomes after mismatched umbilical cord blood transplantation for hematologic malignancy. *Biol Blood Marrow Transplant*.. 2012 Dec 1; 18(12):1890-1896. doi:10.1016/j.bbmt.2012.07.010. Epub 2012 Jul 17. PMC3826155.
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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

relapse by donor activating KIR2DS1. New England Journal of Medicine. 2012 Aug 30; 367(9):805-816. doi:10.1056/NEJMoa1200503. Epub 2012 May 1. PMC3767478.

One manuscript was submitted:

1. Sarah Cooley, et al., The protective effect of unrelated donors with killer-cell immunoglobulin-like recepto[r (KIR) B genes is enhanced in recipients with HLA-C1 group ligands. Submitted to Blood.

Six abstracts were accepted and presented:

1. Fabio Giglio, et al., KIR3DL1/S1 and HLA-B alleles combine to influence unrelated hematopoietic stem cell transplantation outcomes. Oral presentation 2012 BMT Tandem Meetings.
2. Carolyn Hurley, et al., Impact of unidirectional mismatches on the outcome of unrelated donor hematopoietic stem cell transplantation. Oral presentation 2012 IHIW/EFI/BSHI joint meetings.
3. Vanderson Rocha et al., Effect of HLA-matching recipient to donor non-inherited maternal antigens on outcomes after mismatched umbilical cord blood transplantation for hematologic malignancy. Presented at the 10th Annual Cord Blood Symposium, June 7-9, 2012, San Francisco.
4. Lawrence Petz, et al., The cure of HIV infections using cord blood transplantation. Submitted BBMT. Oral presentation 2012 BMT Tandem Meetings.
5. Carolyn Hurley, et al., Impact of unidirectional mismatches on the outcome of unrelated donor hematopoietic stem cell transplantation. Oral presentation 2012 IHIW/EFI joint meetings.
6. Minoo Battiwalla, et al., HLA DR15 antigen status does not impact graft-versus-host disease or disease-free survival in HLA-matched sibling transplantation for hematologic disease. Poster presentation 2011 ASH meeting

**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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3. Eberhard HP, Madbouly AS, Gourraud PA, et al. Comparative validation of computer programs for haplotype frequency estimation from donor registry data. *Tissue Antigens* 2013; 82(2): 93-105.
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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

Patients with High Risk (HR) Multiple Myeloma (MM). Biol Blood Marrow Transplant 2013; 19(2s): S154. Abstract 80.

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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

**Attachment B – Published Manuscripts and Abstracts**  
**Associated with this Grant**

**Manuscripts and Book Chapters**

1. King RJ, Confer DL, Greinix HT, Halter J, Horowitz M, Schmidt AH, Costeas P, Shaw B, Egeland T. Unrelated hematopoietic stem cell donors as research subjects. *Bone Marrow Transplantation* 2011;46:10-13.
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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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**National Marrow Donor Program® N00014-11-1-0339**  
**HLA Typing for Bone Marrow Transplantation**  
**FINAL REPORT**  
**January 1, 2011 – December 31, 2012**

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**FINAL REPORT**  
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**FINAL REPORT**  
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