The Breadth and Depth of CIBMTR: CIBMTR Update
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CIBMTR-Today

CIBMTR is a research collaboration between the NMDP/Be The Match and Medical College of Wisconsin
Purpose

CIBMTR collaborates with the global scientific community to advance hematopoietic cell transplantation and cellular therapy research worldwide to increase survival and enrich quality of life for patients.

We facilitate critical observational and interventional research through our scientific and statistical expertise, our large network of transplant centers and our unique and extensive clinical database.
Locations

• Two campuses; One organization:

• CIBMTR-Milwaukee
  – Medical College of Wisconsin

• CIBMTR-Minneapolis
  – NMDP
Sharing Knowledge

• Key Functional Areas:

- Data Operations
- Information Technology (CIT)
- Statistics and Observational Research
- Clinical Trials Support
- Audit and Monitoring
- Immunobiology Research

Integrated Scientific Oversight
Data Operations

• Manages collection of recipient outcome data from a network of >300 transplant centers
• Manages collection of donor data from all NMDP network DCs, ACs, CCs
• Creates outcomes reports for CBB
• Develops training programs for network staff submitting data
• Performs data entry /imaging functions for research and operational data
Information Technology (CIT)

- FormsNet
  - Electronic data capture system
  - Report management
  - Clinical trials management
  - Auditing and monitoring

- AGNIS
  - Electronic messaging system

- Research Database
  - Study retrievals
  - DBtC
  - Website support
Statistics and Observational Research

• Manages a portfolio of >200 studies across 15 Working Committees
  – Develops protocols
  – Performs analyses
  – Oversees manuscript preparation

• Other analyses
  – HRSA deliverables across all contracts
  – FDA IND annual progress reports
  – DPSM Advisory Group
  – Corporate Studies
  – Info Requests
Clinical Trials Support

• RCI BMT
  – Provides services to investigators conducting phase 1 or 2 multi-site trials
  – Manages PBSC & unlicensed CB FDA INDs
  – Manages MDS CED protocol

• Survey Research Group
  – LTDFU, RDSafe, Financial Impact

• Corporate Studies

• BMT CTN
Audit and Monitoring

• Conducts source document on-site audits for recipient outcomes data

• Monitors clinical trials
  – PBSC FDA IND
  – Unlicensed CB IND
  – RCI BMT clinical trial monitoring
Immunobiology Research

• Oversees Research Sample Repository
• Supports studies utilizing clinical outcomes data and research samples
• Supports BMT CTN and RCI BMT trials
• Supports NMDP Histocompatibility & Cord Blood Advisory Groups, & Bioinformatics Research Advisory Ginger Group
• Represents CIBMTR in International Histocompatibility Working Group
2013 Publications

Outcomes Research: 69%

- BMTCTN: 6%
- Health Services Research: 5%
- Statistical Methodology: 7%
- Bioinformatics: 3%
- Other: 10%
2013 American Society of Hematology Meetings

• 25 Abstracts (17 oral and 8 Poster)
  – 2 Acute Leukemia WC
  – 1 Chronic Leukemia WC
  – 1 Donor Health & Safety WC
  – 3 Graft Sources & Manipulation WC
  – 2 GVHD WC
  – 1 Health Services WC
  – 6 Immunobiology WC
  – 1 Late Effects & Quality of Life WC
  – 2 Lymphoma WC
  – 1 Non-Malignant Marrow Disorders WC
  – 1 Plasma Cell Disorders WC
  – 1 Statistical Center
  – 1 BMT CTN
  – 1 HSR
  – 1 RCI BMT
2014 BMT Tandem Meeting

- 16 Abstracts (13 oral and 3 Poster)
  - 1 Acute Leukemia WC
  - 1 Donor Health WC
  - 1 Health Policy
  - 3 Immunobiology WC
  - 2 Infection WC
  - 1 Lymphoma WC
  - 1 Late Effects
  - 1 Pediatric Cancer WC
  - 5 Regimen-Related Toxicity WC
2013 Research Article Summaries

• 16 lay summaries of research articles published online for patients and family members
Recipient data submitted by centers is essential to CIBMTR research programs:
  - Outcomes research
  - Immunobiology research
  - Health Services research
  - RCI BMT research
  - BMT CTN research

Data used for:
  - Study planning, e.g. how fast can we accrue?
  - Answer protocol research questions
Donor Telomere Length Predicts Survival in Bone Marrow Failure

Shahinaz Gadalla, M.D., Ph.D.
National Cancer Institute
Telomere Length in Hematopoietic Cell Transplantation (HCT)

• Post-HCT telomere shortening has been reported in several studies
  – Maximum shortening in the first year
• In allogeneic transplant recipients, short telomeres were associated with:
  – Female gender
  – Chronic GvHD
  – Older donors
Study Hypothesis

- Pre-HCT leukocyte telomere length is a predictive marker for outcomes after HCT
  - Recipients
  - Donors
Recipient Pre-Transplant Telomere Length Does Not Predict Survival

Log-rank p = 0.8

Short Telomeres (<=25th percentile for age)

Long Telomeres (>25th percentile for age)
Donor Telomere Length Predicts Survival in Bone Marrow Failure Patients

Long Telomeres (T/S>0.8)

Short Telomeres (T/S<=0.8)

Log-rank p = 0.01
Summary & Conclusions

• Short telomeres in bone marrow failure patients had no effect on their post HCT survival

• Longer donor telomeres are associated with better survival outcome
  – Independent of donor age
  – Not altered by recipients’ age, or conditioning regimen

• If replicated, donor telomere length may play a role in guiding donors’ selection
RDSafe Study
Michael Pulsipher, M.D.
University of Utah

CIBMTR
A research collaboration between the National Marrow Donor Program (NMDP)/Be The Match and the Medical College of Wisconsin
RDSafe Study Overview

• Multicenter, observational prospective study evaluating the medical and psychological toxicities of stem cell donation occurring during the first year after collection of related PBSC or BM donors

• Ancillary Health-related Quality of Life (HRQoL) Study
Primary Objectives

• Compare incidence of serious and severe adverse events in related HSC donors in donors 18-40 and 41-60 vs. comparative age groups in cohort of unrelated HSC donors

• Describe incidence and severity of adverse events in related donors under 18 and over 60
RDSafe Study

• Opened to accrual January 2010
• Adult accrual closed May 2013 with 1512 adult donors (>18 yrs.)
• Pediatric cohort anticipated to reach accrual Jan. 2015 with 325 Pediatric donors (5 to 17 yrs.)
Study Methods

- Pre-donation comorbidities and health status submitted by transplant center
- Post-donation follow-up conducted by CIBMTR Survey Research Group
- Donors contacted donor at:
  - One, six and 12 months after donation
- Asked detailed information on
  - Pain levels and,
  - 12 frequently noted symptoms; e.g., nausea, vomiting, insomnia
RDSafe Clinical Study - 2014 Tandem
Abstract

• Abstract ID number: 4144
• Title of the abstract: Related PBSC Donors Age >60 Have High Rates of Baseline and Donation-Related Pain and Slow Recovery: First Report from the Related Donor Safety Study (RDSafe)
• Name of presenting author: Michael Pulsipher
• Assigned session: Oral Abstracts - Session N - Allogeneic Transplants
• Session date and time: Sunday, March 2, 2014, 10:30 AM
Ancillary HRQoL Study

- Study conducted at University of Pittsburgh
- Galen Switzer, Ph.D. Principal Investigator
- Closed to accrual with
  - 99 unrelated donors
  - 314 related donors
Primary Objectives - Ancillary HRQoL Study

- Compare HRQoL between related and unrelated BM and PBSC donors age 18-60
- Compare HRQoL of related pediatric (5-17), adult (18-60) and older adult (>60) donors with age-matched health normative cohorts
- Compare donor experience with clinical status/outcome of their recipient using data collected via the CIBMTR
HRQoL Study Methods

• Structured telephone interviews pre-donation and 4 weeks post-donation

• Interviews focused on
  – Socio-demographics
  – Physical and mental health status
  – Donation-related perceptions
HRQoL - 2013 ASH Abstract

• Critical Finding
  – Despite having somewhat poorer overall general health that is likely age-related, older donors (>60 yrs.) experience similar – and in some domains better – donation-related HRQoL compared to younger related or unrelated donors (18 – 60 yrs.)
Conclusion

• The data that you provide is the foundation of all CIBMTR research programs
• CIBMTR impacts clinical decisions
• Thank you for your diligent oversight and hard work