

# ASBMT eNews

AMERICAN SOCIETY FOR BLOOD AND MARROW TRANSPLANTATION

August 2011

## CLINICAL RESEARCH

### Survival Rates Indicate Benefit to Pediatric Patients with Very High-Risk Leukemias Undergoing Hematopoietic Stem Cell Transplants

Children with very high-risk leukemias benefit from a hematopoietic stem cell transplant early in their diagnosis or relapse treatment regardless of their donor source, according to a *Blood* study. Researchers from St. Jude Children's Research Hospital compared 83 children who received a stem cell transplant for treatment of acute lymphoblastic leukemia (ALL) or acute myeloid leukemia (AML) to 107 children with the same diagnosis who had received treatment and a transplant in previous years. They learned that the five-year survival rates were 65 percent for the ALL patients and 74 percent for the AML patients in the recent group compared with 28 percent and 34

percent, respectively, for the group treated earlier, leading them to conclude that transplant approaches and supportive care have improved and are beneficial, irrespective of the increased use of non-HLA identical donor sources in recent years. [More...](#)

### Transplants Using Double Umbilical Cord Blood or Half-Matched Related Bone Marrow Donors Show Promise for Leukemia and Lymphoma Patients

Two phase 2 studies conducted simultaneously by the Blood and Marrow Transplant Clinical Trials Network (BMT CTN) show promise that unrelated double umbilical cord blood (dUCB) or haploidentical related donor bone marrow can be used successfully for hematopoietic stem cell transplants after reduced intensity conditioning.

*Continues on page 4*

## ASSOCIATION NEWS

### Call for Nominations for *Biology of Blood and Marrow Transplantation* Editor-in-Chief

The ASBMT Publications Committee is requesting nominations for the position of Editor-in-Chief of *Biology of Blood and Marrow Transplantation* (BBMT) to begin Jan. 1, 2013. Any ASBMT member may self-nominate or submit a name for nomination. The nominee should approve the submission of his/her name. [More...](#)

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## A WORD FROM PRESIDENT DANIEL WEISDORF, MD

### Can We Do More for Less?: Standardization to Extend Our Reach

Hematopoietic cell transplantation is an expensive proposition. We use lots of tests, drugs, imaging and transfusions; and the follow-up period extends for some time. While current approaches have improved survival, reduced morbidity, limited transfusions and shortened hospital stays, we have also added high-priced imaging, extended spectrum antimicrobials, and molecular monitoring for infection, relapse and chimerism. Even our graft sources cost more: one unrelated donor graft or a pair of umbilical cords can cost \$30,000 to \$60,000. Chronic GVHD, extended immunosuppression and infection vulnerability requires active monitoring and costly care for several years.

However, many patients are still not getting the curative transplants they need. Nearly all patients with AML and most adults with ALL need an allograft to live beyond three years and most with recurrent lymphoma need either an auto or an allograft. Some contend that nearly all patients with symptomatic myeloma would benefit from autografting and extended maintenance therapy. Each of these broader transplant applications can cure more patients, extend their survival and improve their life's quality, but at substantial cost. The National Marrow Donor Program (NMDP) in a System Capacity Initiative has challenged the international community to develop techniques and resources to perform 20,000 unrelated transplants by 2020 – quadrupling this year's number. The need for physicians and other providers, facilities, transfusion products and even data structures to capture this growing effort are all daunting. This multiyear initiative is addressing the multiparameter challenges that this stretch goal demands.

One possible approach might be standardization in delivery of care. I've been struck by the extreme variability in all of our habits. While many agree on a transfusion trigger of 10,000 platelets, we may differ in how often a non-infected, minimally symptomatic neutropenic outpatient autograft or reduced intensity allograft recipient should be seen. Are seven, five or two visits per week sufficient to check their status, plan their transfusions and to keep them safe? Does it differ for educated, highly motivated medical consumers with a hovering caregiver noting every symptom? Should older, frailer patients be seen more? If patients and families are sufficiently

educated (perhaps supported by a checklist) can they safely monitor the aspects of history and review of systems that we routinely do in clinic visits? Could telephone contact substitute in asking those same questions?

Yet monitoring by phone, patient and family report means taking responsibility for the patient's safety – but with no professional fees attached. Remote monitoring could cut lab testing by 50 percent to 75 percent, but truncate the institutional revenue that underwrites the needed support systems for these “easier” transplants. Some autograft recipients are hospitalized until neutrophil recovery – either by habit, physician comfort or lesser access to outpatient care and transfusion support. In some institutions, reduced intensity allografts (who rarely get mucositis, VOD or early pneumonitis) stay hospitalized because of habitual daily visits and round-the-clock nursing observation even though family and patient report might suffice. In some clinics, CBC, electrolytes and magnesium are monitored daily, liver function thrice weekly, and CMV, cyclosporine or tacrolimus levels weekly. Reducing lab testing, surveillance cultures, scheduled imaging or overly frequent PCR surveillance might indeed cut costs, but best practices for how many visits, which testing and at what frequency remain undefined.

Some people just order more tests. Some – often younger faculty and midlevel providers – offer exquisitely compulsive care. Does their thoroughness provide a measure of safety superior to that which older physicians provide through eyeball testing and more confident judgement? Alternatively, does experience blur confidence into complacency? Providers of different age, background and personality give different care: not better or worse, just different.

Perhaps standardization using consensus surveillance and management practices could limit variation, improve care and, thereby, limit cost.

Could we study how care can be just as safe with fewer visits, testing, imaging and drugs – perhaps a bit less of everything? Maybe as our field matures and we all get wiser, we will learn how to do more ... with much less. -Daniel



## ASSOCIATION NEWS (CONTINUED FROM PAGE 1)

### Free ASBMT Membership for Trainees

Postdoctoral fellows and physicians-in-training for blood and marrow transplantation are eligible for free membership in the American Society for Blood and Marrow Transplantation. From August through October, annual dues are waived for new trainees who apply for membership in the Society. The program is made possible through a grant from Otsuka America Pharmaceuticals, Inc. [More...](#)

### Oct. 1 Deadline for New Investigator Awards

New investigator awards of \$60,000 each, to be presented at the 2012 BMT Tandem Meetings, are being supported by Amgen, Genentech, Millennium, Otsuka and ASBMT. The deadline for applications is Oct. 1. [More...](#)

### NHLBI/PACT Workshop Announcement

The NHLBI/PACT Workshop “Cell Therapy for Pediatric Diseases: A Growing Frontier” will take place Sept. 14-15 at the Lister Hill Center Auditorium of the National Library of Medicine in Bethesda, Md. This workshop will address strategies to overcome the barriers to advancing the development and delivery of cell-based therapies for pediatric patients, in particular those with rare and life-threatening diseases. The clinical applications of cellular therapies and regenerative medicine, including the ethical considerations and models of clinical trial design, will be examined with intent to optimize overall processes for the future. Visit [www.pactgroup.net](http://www.pactgroup.net) for workshop details.

### Immune Tolerance Network

Immune Tolerance Network (ITN) is a clinical research consortium dedicated to the development of immune tolerance therapies for autoimmune disease and transplantation. The ITN is currently seeking short “Concept Proposals” for novel clinical trials designed to induce immune tolerance in combined allogeneic

solid organ transplantation plus therapeutic cell transfer. The proposal submission deadline is Aug. 31. [More...](#)

### Call for Clinical Trial Contributions

The Federation of Clinical Immunology Societies (FOCIS) has created an opportunity for collaboration with their member societies in building an interdisciplinary clinical trial listing in the new ePublication, *Translational Immunology Update*. The section will include phase 1 and 2 studies using novel immunotherapeutics or standard immunotherapeutics in new diseases or new ways. The trials will be formatted in a table with key variables including the drug name, disease, N, overall study design, primary outcome variables and results. The goal is for readers to have a better understanding of the application of clinical immunology outside of their field. To participate, submit trials that you are aware of to

<http://translationalimmunology.pbworks.com/Clinical-Trial-Contributions>.

### Summit on Cell Therapy for Cancer

The Summit on Cell Therapy for Cancer will include dynamic discussions and lectures by leaders in the field to provide an in-depth review of the state-of-the-art cell therapy as a cancer immunotherapy. The 1½-day program will include presentations on the latest research on induced pluripotent stem cells, reprogramming immune cells and translational considerations, various cell therapy modalities, regulatory considerations, methods for T cell expansion, biomarkers, adoptive immune therapies for melanoma, persistence of transferred cells, differentiation of human and mouse immune stem cells, and clinical trials that will impact cell therapies from the perspective of the Cancer Immunotherapy Trials Network. For more information on the program, speakers and registration, please visit

<http://www.sitcancer.org/meetings/am11/summit11/>

## CLINICAL RESEARCH (CONTINUED FROM PAGE 1)

Comparing 100 adult patients with leukemia or lymphoma, half of the group received haploidentical related bone marrow transplants (BMT CTN 0603) and the other half received unrelated dUCB transplants (BMT CTN 0604). The one-year probabilities of progression-free survival were 46 percent for cord blood

transplant recipients and 48 percent for half-matched, related bone marrow recipients, according to the study appearing in *Blood*. A more extensive randomized clinical trial is expected to test the relative efficacy of these two transplant approaches. [More...](#)

## BASIC SCIENCE STUDIES

### Purified Stem Cell Can Regenerate Blood System

For the first time ever, researchers were able to purify a single hematopoietic stem cell capable of regenerating the entire blood system. CD 49f was identified by researchers at the Ontario Cancer Institute and University of Toronto as a specific marker in the cells that turned out to be highly efficient at producing long-term multilineage grafts. Appearing in *Science*, the study may prove useful for developing stem-cell based therapeutics to treat a multitude of diseases. [More...](#)

### DNA Encoding Determines Cell Type from Onset

From the beginning, human embryonic stem cells know what type of specialized cell they are going to become, according to a new study published in *Cell Stem Cell*. Researchers from McMaster University discovered that histone modifications of DNA in a pluripotent stem cell determine pluripotency vs. lineage differentiation. The scientists also learned that when the cells were forced to become a blood or neural cell, five times the blood cells and 12 times the neural cells were generated. [More...](#)

## BMT TANDEM MEETINGS

### Registration Open for 2012 BMT Tandem Meetings in San Diego

Online registration and housing is now open for the 2012 BMT Tandem Meetings Feb. 1-5 in San Diego. Links to meeting registration, housing reservations, preliminary program, abstract submission and parallel conferences can all be found in one convenient location. [More...](#)

### Abstract Submission Deadline is Oct. 13 for Tandem Meetings

Abstracts for the BMT Tandem Meetings in San Diego will be accepted through Oct. 13. Invitations for oral presentations will be offered to more than 100 authors whose abstracts received the highest scores from the review committees. Many others will be accepted for poster presentations. [More...](#)

## BREAKING NEWS

Breaking news in the pharmaceutical industry can be found in the following news releases: ["Chimerix Commences Phase 2 Clinical Study of CMX001 for Prevention of Adenovirus Disease in Pediatric and Adult Hematopoietic Stem Cell Transplant Patients"](#) and ["Vical and Astellas Announce Worldwide License Agreements for TransVax Cytomegalovirus Vaccine."](#)

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## CALENDAR OF EVENTS

### •AUGUST

#### **2011 Pan Pacific Lymphoma Conference**

August 15-19  
Koloa, Kauai, Hawaii

#### **Society for Hematology and Stem Cells**

40<sup>th</sup> Annual Scientific Meeting  
August 25-28  
Vancouver, Canada

### •SEPTEMBER

#### **2<sup>ND</sup> Donor Outcome Workshop**

September 1-2  
Leiden, The Netherlands

#### **BMT InfoNet**

4<sup>th</sup> National Survivorship Conference  
September 10-11  
Atlanta, Georgia

#### **ISCT North American Regional Meeting**

September 15-16  
Charlottesville, Virginia

#### **ESH-ICMLF 13<sup>th</sup> International Conference on Chronic Myeloid Leukemia – Biological Basis of Therapy**

September 22-25  
Estoril, Portugal

#### **European Society for Medical Oncology**

The European Multidisciplinary Cancer Congress  
September 23-27  
Stockholm, Sweden

### •SEPTEMBER (CONT.)

#### **REACT/TS 5<sup>th</sup> International Symposium**

September 27-29  
Miami, Florida

### •OCTOBER

#### **American Society for Histocompatibility and Immunogenetics**

37<sup>th</sup> Annual Meeting  
October 17-21  
New Orleans, Louisiana

#### **American Association of Blood Banks**

2011 Annual Meeting  
October 22-25  
San Diego, California

#### **World Cord Blood Congress**

October 27-29  
Rome, Italy

#### **European Society of Gene & Cell Therapy**

19<sup>th</sup> Annual Congress  
October 27-31  
Brighton, United Kingdom

#### **ISCT Australasia Regional Meeting**

October 30-November 2  
Bunker Bay, Australia

### •NOVEMBER

#### **2011 World Conference on Regenerative Medicine**

November 2-4  
Leipzig, Germany

### •NOVEMBER (CONT.)

#### **National Marrow Donor Program**

2011 Council Meeting  
November 3-5  
Minneapolis, Minnesota

### •DECEMBER

#### **American Society of Hematology**

53<sup>rd</sup> Annual Meeting  
December 10-13  
San Diego, California

### •JANUARY

#### **Phacilitate Cell & Gene Therapy Forum 2012**

January 30-February 1  
Washington, D.C.

### •2012

#### **BMT Tandem Meetings**

Combined ASBMT and CIBMTR Annual Meetings  
February 1-5  
San Diego, California

### •2013

#### **BMT Tandem Meetings**

Combined ASBMT and CIBMTR Annual Meetings  
February 13-17  
Salt Lake City, Utah

### •2014

#### **BMT Tandem Meetings**

Combined ASBMT and CIBMTR Annual Meetings  
February 19-23  
Orlando, Florida

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