Penn Medicine

CLINICAL BRIEFING™

Abramson Cancer Center • Hematologic Malignancies Bone Marrow Transplant

Enrolling Clinical Trials: Umbilical Cord Blood Transplantation vs Haploidentical Transplantation for Patients with Hematological Malignancies

Researchers at Penn Medicine and the Abramson Cancer Center are participating in a multicenter study to compare double umbilical cord blood (dUCB) transplantation and haploidentical cell transplantation (haplo-BM) for the treatment of patients with hematological malignancies.

For persons with blood cancers at risk of relapse, allogeneic hematopoietic stem cell transplantation offers the best chance for a durable response. Related matched donors, typically siblings, are the ideal source of stem cells, but only one in four will be an HLA-identical match. Thus, a significant proportion of patients will not have a sibling donor, and will receive stem cells from other sources.

At the Abramson Cancer Center, the primary source of stem cells outside of related matched donors are HLA-matched unrelated donors, who are accessed through the National Marrow Donor Program (BE THE MATCH)TM. Matched unrelated donors can provide a solid HLA-match, but the time required to identify and accurately pair donor and recipient can exceed the time to disease progression and up to a third of patients won't find a suitable match (i.e., no more than a mismatch at a single locus).

To address these issues and expand the potential donor pool for hematological stem cell transplantation, the Abramson Center offers access to two alternative sources, double-UCB (dUCB) and half-matched related (HLA-haploidentical) bone marrow donors. The use of two units of umbilical cord blood increases the number of cord blood cells to improve the success of engraftment.

Both UCB and haploidentical SCT are associated with unique advantages and applications, and these are the source of inquiry for an ongoing multicenter comparative clinical trial currently enrolling patients at Penn Medicine. The trial is being conducted under the aegis of the NIH-funded Blood and Marrow Transplant Clinical Trials Network (BMT CTN).

This study follows previous investigations that evaluated the safety and efficacy of related haploidentical and dUCB transplantation after reduced intensity conditioning (RIC), a regimen that uses less chemotherapy and radiation than standard myeloablative conditioning. In these trials, both UCB and haploidentical BMT produced early results similar to that reported for HCT with unrelated donors.

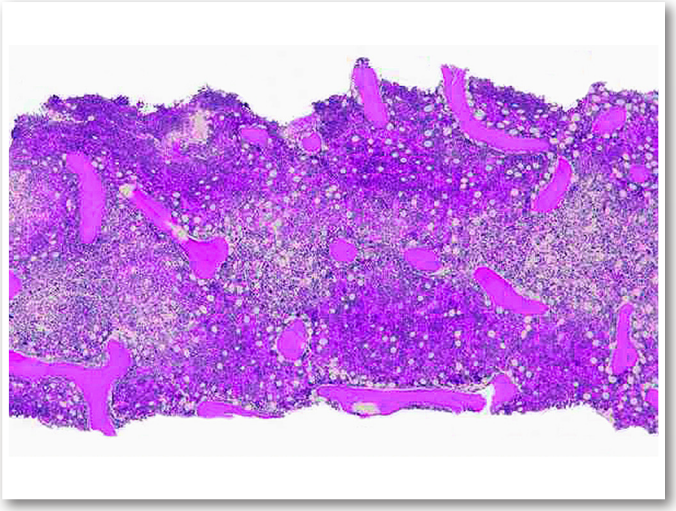

Figure 1: Acute myelogenous leukemia demonstrating greater than 20% myeloblasts in the marrow and greater than 10% mature myeloid elements beyond the blast stage.

Double Cord Versus Haploidentical (BMT CTN 1101) [NCT01597778]

Objective: This study will test the hypothesis that progression free survival at two years after RIC haplo-BM transplantation is similar to the progression free survival after RIC dUCB transplantation.

Methods: Patients with leukemia or lymphoma will be randomized to receive two units of UCB or haploidentical transplant. Patients in both arms of the study will receive reduced intensity conditioning regimens including total body irradiation, as well as GVHD prophylaxis.

Endpoints: The primary endpoint is progression-free survival (PFS) at 2-years from the date of randomization. PFS is defined as the time interval from date of randomization and time to relapse/progression, to death or to last follow-up. Secondary endpoints will assess the success of donor cell engraftment, platelet and neutrophil recovery and acute and chronic graft-vs-host-disease. Overall survival and treatment-related mortality will also be assessed.

Inclusion/Exclusion: Patients with any of the following conditions may enroll: ALL or AML in first complete remission (CR) not considered favorable-risk; acute leukemias in second or subsequent CR; biphenotypic/undifferentiated/prolymphocytic leukemias in first or subsequent CR; adult T-cell leukemia/lymphoma in first or subsequent CR; Burkitt's lymphoma in second or subsequent CR; chemotherapy-sensitive lymphoma. Patients must have adequate cardiac, hepatic, renal and pulmonary function.

Patients with a suitably matched related or unrelated donor, as defined per institutional practice are ineligible. Patients with chronic lymphocytic leukemia (CLL) are not eligible regardless of disease status.

Interested persons are encouraged to contact Elizabeth Hexner, MD, at elizabeth.hexner@uphs.upenn.edu.

FACULTY TEAM

Investigators with Penn Hematology/Oncology are focused on translating laboratory work into novel therapies and practice-changing discoveries. The scope of Penn's hematology and medical oncology clinical research enterprise is very broad, spanning all phases of clinical research, including pre-clinical work and discovery, phase 1 and 2 studies and leadership of national phase 3 trials intended to change the standard of care.

Penn clinical investigators regularly publish high profile and important findings in diverse fields, ranging from the most fundamental cellular investigations, to leading edge translational and clinical research.

Conducting Clinical Studies in Hematological Malignancies at Penn Medicine and the Abramson Cancer Center

Adam D. Cohen, MD
Director, Myeloma Immunotherapy
Assistant Professor of Medicine

Noelle Frey, MD
Assistant Professor of Medicine

Alfred Garfall, MD
Assistant Professor of Medicine

Saar I. Gill, MD, PhD
Assistant Professor of Medicine

Elizabeth O. Hexner, MD
Assistant Professor of Medicine

Rebecca L. Hirsh, MD
Assistant Professor of Clinical Medicine

James A. Hoxie, MD
Professor of Medicine

Peter S Klein, MD, PhD
Professor of Medicine

Daniel Landsburg, MD
Assistant Professor of Clinical Medicine

Alison Loren, MD, MSCE
Associate Professor of Medicine

Selina M. Luger, MD
Director, Leukemia Program
Professor of Medicine

James K. Mangan, MD, PhD
Assistant Professor of Clinical Medicine

Anthony R. Mato, MD, MSCE
Assistant Professor of Medicine

Sunita Nasta, MD
Associate Professor of Clinical Medicine

Alexander E. Perl, MD
Assistant Professor of Medicine

David L. Porter, MD
Director, Blood and Marrow Transplantation
Jodi Fisher Horowitz Professor in Leukemia Care Excellence

Stephen J. Schuster, MD
Robert and Margarita Louis-Dreyfus Associate Professor in Chronic Lymphocytic Leukemia and Lymphoma Clinical Care and Research

Edward Stadtmauer, MD, (left) and the research support staff of the Hematologic Malignancies Bone Marrow Transplant program at the Abramson Cancer Center.

Edward A. Stadtmauer, MD
Section Chief, Hematologic Malignancies
Professor of Medicine

Jakub Svoboda, MD
Assistant Professor of Medicine

Donald Tsai, MD, PhD
Associate Professor of Medicin

Daniel Vogl, MD
Assistant Professor of Medicine

Brendan M. Weiss, MD
Assistant Professor of Medicine

Non-Physician Providers

Sarah J. Del Percio, MSN, CRNP
Bernadette B. Diccion, MSN, CRNP
Heather DiFilippo, MSN, CRNP
Colleen Harker Erb, MSN, CRNP
Danielle Land, MSN, CRNP
Patricia Mangan, MSN, CRNP
Mary Sanchez, MSN, CRNP
Brenda Shelly, MSN, CRNP
Jacqueline Smith, MSN, CRNP
Brandi Swisher, MSN, CRNP

ACCESS

Perelman Center for Advanced Medicine
2 West Pavilion
3400 Civic Center Boulevard
Philadelphia, PA 19104

Cancer Clinical Trials at Penn Medicine

For information regarding cancer clinical trials at Penn Medicine, please visit the Oncolink Clinical Trial Matching and Referral Service at:
www.oncolink.org/treatment/trials.html.

Physician Liaison, Cancer & Surgery

Donna Fenske Seeger
Cell: 215-410-7472

FY-16 9815 03.16