

CIBMTR Contact:

Liz Siepmann, Communications Specialist esiepmann@mcw.edu

Results of multicenter clinical trial sponsored by National Marrow Donor Program /Be The Match to be published in Journal of Clinical Oncology

Approach for mismatched, unrelated bone marrow donors could expand transplant options for racially/ethnically diverse patients

Minneapolis, Minn., April 27, 2021 – The CIBMTR® (Center for International Blood & Marrow Transplant Research®) manuscript entitled, "A National Marrow Donor Program Sponsored Multi-Center, Phase II Trial of HLA-Mismatched Unrelated Donor Bone Marrow Transplantation Using Post-Transplant Cyclophosphamide," has been published in the <u>Journal of Clinical Oncology</u>.

The CIBMTR is a research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin (MCW). Dr. Bronwen Shaw, Chief Scientific Director of CIBMTR, MCW, protocol co-chair of the clinical trial and Professor at MCW in Milwaukee, WI, is first author of the manuscript, which details the results of a prospective clinical trial to assess the outcomes of mismatched, unrelated donor (MMUD) transplantation using post-transplant cyclophosphamide (PTCy) with bone marrow grafts.

The trial is the largest prospective multicenter study of this approach in human leukocyte antigen (HLA) MMUD transplantation to date. It successfully achieved its primary objectives, with high rates of overall survival after MMUD bone marrow transplantation using PTCy, sirolimus and mycophenolate mofetil to prevent graft-versus-host disease.

"For patients who lack a well-matched donor, especially ethnically diverse patients, this approach could bridge a gap in access to transplant and make the likelihood of donor availability almost 100%," said Dr. Shaw.

Almost 50% of the 80 patients enrolled in the trial at 11 U.S. transplant centers were racial/ethnic minorities who did not have an available well-matched donor in their family or any unrelated donor registry. The trial provides compelling evidence that this treatment approach using MMUD is a good option for patients who do not have a well-matched family or unrelated donor.

Blood and bone marrow cell transplant is a well-established and potentially curative therapy for hematological diseases, and the best outcomes are often seen in the setting of a well-matched related or unrelated donor using well established historical transplant approaches. Patients from racial/ethnic minorities are disproportionately disadvantaged in access to transplant, as the likelihood of finding a well-matched unrelated donor is significantly less than for white patients.

"Having strategies for safe, effective transplant using MMUD expands access to a potentially curative therapy to all patients in need," said Dr. Shaw. "We're pleased to have this study featured in the *Journal of Clinical Oncology* so that our findings about this good option for transplant can be shared widely, especially to those doctors who refer patients to transplant centers, for the benefit of patients everywhere."

The study was also presented as an <u>oral abstract</u> at the 62nd American Society of Hematology Annual Meeting in December, 2020.

In February, NMDP/Be The Match announced plans to launch a follow-up study. The ACCESS clinical trial, "A Multicenter Phase II Trial of HLA-Mismatched Unrelated Donor Hematopoietic Cell Transplantation with Post-Transplantation Cyclophosphamide for Patients with Hematologic Malignancies," using peripheral blood stem cells (PBSC) as the graft source will greatly expand the reach of this approach because the clear majority of allogeneic transplants for adult patients use PBSC grafts. The ACCESS protocol also will explore the safety and efficacy of MMUD BM in pediatric patients with hematological malignancies who lack well matched family and unrelated donor options.

According to Dr. Steven Devine, Chief Medical Officer at NMDP/Be The Match® and Associate Scientific Director of CIBMTR, the upcoming ACCESS study "will seek to replicate or improve upon the results observed in the prior MMUD using a simpler method to obtain cells from donors (PBSC) and will expand access to MMUD transplantation for pediatric patients with limited alternative treatment options." The ACCESS study is set for activation later in 2021.

About the CIBMTR

The CIBMTR® (Center for International Blood and Marrow Transplant Research®) is a research collaboration between the National Marrow Donor Program® (NMDP)/Be The Match® and the Medical College of Wisconsin (MCW). The CIBMTR collaborates with the global scientific community to advance hematopoietic cell transplantation (HCT) and cellular therapy worldwide to increase survival and enrich quality of life for patients. The CIBMTR facilitates critical observational and interventional research through scientific and statistical expertise, a large network of transplant centers, and a unique and extensive clinical outcomes database. For more information on the CIBMTR, please visit www.cibmtr.org or follow the CIBMTR on Facebook, LinkedIn, or Twitter at @CIBMTR.

About the Medical College of Wisconsin

With a history dating back to 1893, The Medical College of Wisconsin is dedicated to leadership and excellence in education, patient care, research and community engagement. More than 1,400 students are enrolled in MCW's medical school and graduate school programs in Milwaukee, Green Bay, and Central Wisconsin. MCW's School of Pharmacy opened in 2017. A major national research center, MCW is the largest research institution in the Milwaukee metro area and second largest in Wisconsin. In the last ten years, faculty received more than \$1.5 billion in external support for research, teaching, training and related purposes. This total includes highly competitive research and training awards from the National Institutes of Health (NIH). Annually, MCW faculty direct or collaborate on more than 3,100 research studies, including clinical trials. Additionally, more than 1,600 physicians provide care in virtually every specialty of medicine for more than 2.8 million patients annually.

About National Marrow Donor Program/Be The Match

The National Marrow Donor Program® (NMDP)/Be The Match® is the leading global partner working to save lives through cellular therapy. With more than 30 years of experience managing the most diverse registry of potential unrelated blood stem cell donors and cord blood units in the world, NMDP/Be The Match is a proven partner in providing cures to patients with life-threatening blood and marrow cancers and diseases. Through their global network, they connect centers and patients to their best cell therapy option—from blood stem cell transplant to a next-generation therapy—and collaborate with cell and gene therapy companies to support therapy development and delivery through Be The Match BioTherapies®. NMDP/Be The Match is a tireless advocate for the cell therapy community, working with hematologists/oncologists to remove barriers to consultation and treatment, and supporting patients through no-cost programs to eliminate non-medical obstacles to cell therapy. In addition, they are a global leader in research through the CIBMTR® (Center for International Blood and Marrow Transplant Research®)—a collaboration with Medical College of Wisconsin, investing in and managing research studies that improve patient outcomes and advance the future of care.

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Likelihood of donor match almost 100% with approach outlined in clinical trial to be published in JCO