



# Outcomes Research Brings Best Practices to Blood and Marrow Transplantation

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For many patients facing certain cancers and autoimmune diseases, transplantation of blood stem cells from bone marrow, peripheral blood, and/or cord blood represents the best hope for survival. Make no mistake; this is a complex process entailing complete eradication and replacement of the patient's immune system.

With such high stakes, it is critical for physicians to know whether the potential reward outweighs the risks of this treatment and to determine which approach is most effective for each specific disease. Developing high-quality, evidence-based protocols requires high-quality outcomes data. One clinical center could never perform enough transplants to generate the information needed for meaningful outcomes research; however, by collecting results from transplant centers across the world, researchers can accumulate a large enough sample size to inform future treatment decisions.

That was the vision when a Medical College of Wisconsin professor, the late Mortimer Bortin, MD, and his colleagues founded the International Bone Marrow Transplant Registry (IBMTR) in 1972, just 4 years after the first successful hematopoietic cell transplantation. At the time, there were only about 12 transplant centers and fewer than 50 patients a year receiving transplants worldwide.

The scope of this effort today is striking by comparison. First came the addi-

tion of the Autologous Blood and Marrow Transplant Registry in 1990. Then in 2004, the Medical College registries combined with the National Marrow Donor Program's outcome research program to synergize the work of these complementary organizations.

This union created the Center for International Blood and Marrow Transplant Research (CIBMTR), which collaborates with the global scientific community to advance hematopoietic cell transplantation and cellular therapy research worldwide. Housed at the Medical College of Wisconsin, where Chief Scientific Director Mary M. Horowitz, MD, the Robert A. Uihlein Professor in Hematologic Research, provides leadership, the CIBMTR collects outcomes data on every allogeneic blood and marrow transplant as well as many autologous transplants performed in the United States.

Currently, the CIBMTR's clinical database contains information on almost 400,000 autologous, related, and unrelated donor transplant recipients. Supplying this data is our network of 450 transplant centers in almost 50 countries. The Center's prospective and observational research has resulted in more than 500 publications and includes more than 250 active studies.

The CIBMTR is a truly unique resource that, although based in Wisconsin at the Medical College, has the ability to benefit patients everywhere. Among its extensive contributions, CIBMTR research has identi-

fied factors affecting outcomes, such as age, stage of disease, and conditioning regimens; determined efficacy of various donor types and graft sources; and assessed long-term quality of life and late complications after transplantation.

The Center's past is remarkable, but the most significant discoveries may be yet to come. In August, the National Heart, Lung and Blood Institute and the National Cancer Institute awarded the Medical College of Wisconsin a 6-year, \$45 million grant to support the CIBMTR's leadership role in facilitating large prospective clinical trials through the US Blood and Marrow Transplant Clinical Trials Network. It is the largest grant in Medical College of Wisconsin history.

Results of blood and marrow transplantation have improved dramatically since the 1970s, when only about 15% of patients survived. Still, only about half of patients who need a transplant receive one, only half of those who receive a transplant become long-term survivors, and about half of long-term survivors have ongoing medical problems that affect their daily life. We hope this remarkable investment in clinical research will lead to significant strides in donor matching, survival rates, and quality of life.

The overall goal of the grant is to complete high-quality clinical trials that focus on the most important barriers to transplant success. It specifically is funding the Data and Coordinating Center Consortium, which sup-

ports the Clinical Trials Network by managing the efficient development, implementation, and completion of phase I-III clinical trials, including database management, storage of biologic specimens, regulatory compliance, and numerous other oversight responsibilities. The Medical College is the lead institution for the Data and Coordinating Center.

The Clinical Trials Network, established in 2001, has launched more than 25 multi-center trials involving nearly 4000 patients in the United States. Those trials have resulted in 15 published papers with another five in pre-publication. Their findings have had important implications for blood and marrow transplantation practice. For example, one Network trial identified the potential benefit of removing T-cells, which cause graft-vs-host disease, from grafts before transplantation in acute myelogenous leukemia. Another trial – the largest study of transplantation for multiple myeloma ever conducted – determined that 2 sequential autologous transplants gave results similar to an autologous followed by an allogeneic transplant, in contrast to results from previous small-scale studies.

These are significant findings, yet they are the tip of the iceberg. The CIBMTR's unique, collaborative efforts will grow our knowledge base while measuring the success of current treatments and evaluating new therapies for blood and marrow transplantation. Safety, efficacy, best practices, and the best patient outcomes will be the hallmarks of our work.

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