Division of Blood and Marrow Transplantation

The Division of Blood and Marrow Transplantation at Children's National Health System is a nationally recognized leader in pediatric blood and marrow treatment advances, known for its cutting-edge treatment protocols developed by Children's physicians. Since 1988, Children's specialists have performed more than 1,000 blood and marrow transplants (BMT).

The division focuses on caring for children and their families, and has unique strengths in treating inherited immunodeficiencies, sickle cell disease, leukemia, and malignant brain tumors. The team also treats:

- Aplastic anemia
- Fanconi anemia
- Hodgkin lymphoma
- Metabolic or lysosomal storage diseases
- Neuroblastoma and other solid tumors
- Non-Hodgkin lymphoma
- Thalassemia

Children's National offers a range of treatments for children and young adults up to 30 years of age, including allogeneic, autologous (self), and cord blood transplants for malignant and non-malignant pediatric diseases.

The transplant team's physicians, nurses, pharmacists, social workers, chaplains, and physical therapists are focused on delivering care to BMT patients in its state-of-the-art unit, located in Children's East Inpatient Tower. The BMT inpatient unit features private HEPA-filtered isolation rooms with a specialized water system, assuring a high level of protection against infections and creating an environment essential to successful transplantation. The BMT outpatient unit features four isolation rooms with dedicated nursing.

The Division of Blood and Marrow Transplantation is the region's only dedicated pediatric National Marrow Donor Program transplant center, and is a participating center in the National Institutes of Health-funded BMT Clinical Trials Network, the Center for International Blood and Marrow Transplant Research, and the Pediatric Blood and Marrow Transplant Consortium.

It also is a Children's Oncology Group-accredited transplant program. There are a number of national BMT clinical trials led by our physicians and investigators, some of whom are internationally recognized in the field.

There also are several clinical trials for BMT patients offered in conjunction with these organizations and Children's Research Institute, the academic arm of Children's National Medical Center. The Division of Blood and Marrow Transplantation is part of the Center for Cancer and Blood Disorders.

Key Services

Allogeneic Blood and Marrow Transplantation for Malignancies

David Jacobsohn, MD, ScM

Catherine Bollard, MD

Evelio Perez-Albuerne, MD, PhD

Kirsten Williams, MD

Kristen Barbieri, PA-C

Bonnie Yates, CPNP-AC

Blood and marrow transplantation is a special therapy for patients with cancer or other diseases that affect the bone marrow. Allogeneic transplantation is a procedure in which a person receives hematopoietic (blood-forming) or blood stem cells, from a genetically similar, but not identical, donor. Blood stem cells are found in the bone marrow, peripheral blood, or umbilical cord blood. The first allogeneic BMT was performed at Children's in 1988. Children's patients have access to cutting-edge treatments through several clinical trials for allogeneic blood and marrow transplantation.

David Jacobsohn, MD, ScM, leads the national trial through the Pediatric Blood and Marrow Transplant Consortium (PBMTC) examining the role of minimal residual disease (MRD) after transplants for acute myeloid

leukemia (AML) in children. Kirsten Williams, MD, is building on that trial and studies novel ways to treat MRD following transplant, before frank relapse occurs. Catherine Bollard, MD, brings novel cellular therapy approaches for patients with relapse or very high-risk leukemia.

Autologous Blood and Marrow Transplantation

Evelio Perez-Albuerne, MD, PhD

Kristen Barbieri, PA-C

Autologous transplants are the most common type of BMT treatment, and the division performs about 30 per year under the leadership of Evelio Perez-Albuerne, MD, PhD. Autologous BMT at Children's is performed for patients with solid tumors. In this type of transplant, blood-forming stem cells are collected from the patient and stored frozen. At a later time, the patient receives high-dose chemotherapy, and then the previously collected stem cells are thawed and transplanted into the patient. Autologous stem cells are usually collected from the peripheral blood (see the section on Peripheral Blood Stem Cell Collection) but can be collected from the bone marrow, if necessary.

Although Children's oncologists refer many autologous transplant patients, the program also works with oncologists at other hospitals in the region and across the country to provide the transplant-related portion of a patient's treatment. Once that part of the treatment is complete, patients return to the care of their oncologist for either further treatment or follow-up. Children's has pioneered the use of autologous transplants for patients with hard-to-treat brain tumors. The team at Children's wrote one of the first peer-reviewed articles showing that more of these children could be cured with autologous transplants than with conventional treatment plans that did not include transplant. Children's also performs autologous transplants for selected patients with high-risk neuroblastoma, Ewing sarcoma, Hodgkin lymphoma, and other cancers. A manuscript was recently published by Children's National on risk factors for toxicity following autologous transplants.

Allogeneic Blood and Marrow Transplantation for Sickle Cell Disease

Allistair Abraham, MD

Jacqueline Dioguardi, PA-C

The Sickle Cell Disease Program is one of the largest in the country, treating more than 1,200 children annually. Children's Sickle Cell Program is dedicated to finding new and more effective therapies by participating in clinical trials sponsored by the National Institutes of Health (NIH). Early diagnosis and prevention of complications is critical in sickle cell disease treatment, and the multi-disciplinary hematology team offers a variety of therapies. However, blood and marrow transplantation is the only treatment option available today that can cure sickle cell disease. Study results show there is a 95 percent chance a patient will be cured in matched related transplants. Children's also has a number of critical trials open that study transplantation of unrelated donor marrow for patients with sickle cell disease and prior complications. Initial results are very promising. The division performed the first bone marrow transplant for a patient with sickle cell disease in 1996. Children with sickle cell disease undergoing transplant will be seen by a comprehensive program led by Allistair Abraham, MD, our transplant expert dedicated to sickle cell disease BMT. This team also includes hematologists, pulmonologists, and transfusion medicine specialists for the best possible care of these patients.

Allogeneic Blood and Marrow Transplantation for Immunodeficiency and Inborn Errors of Metabolism

Brett Loechelt, MD, PI, Primary Immune Deficiency Treatment Consortium (PIDTC)

Johanna Curtis, NP-C

The BMT program for immunodeficiencies and inborn errors of metabolism is dedicated to therapy and research of these nonmalignant disorders. The program is led by Brett Loechelt, MD, PI, an immunologist trained in blood and marrow transplantation. Children's National is unique in this regard as only a very few institutions in the United States have a physician with this type of specialty training and the only one in the lower Mid-Atlantic region. Dr. Loechelt is a principal investigator with the Primary Immune Deficiency Treatment Consortium (PIDTC), a NIH-sponsored program to investigate primary immune deficiencies and develop new transplant and other treatment strategies for children with primary immune deficiencies. The program specializes in the use of reduced intensity preparative regimens for treatment of these disorders. Immune deficiency diseases treated through this program include severe combine immunodeficiency syndrome (SCID), Wiskott-Aldrich syndrome (WAS), hemophagocytic lymphohistiocytosis (HLH), IkappaBalpha mutation, and reticular dysgenesis. Dr. Loechelt also leads the program transplant for inborn errors in collaboration with the Division of Genetics and Metabolism. Children's Division of Genetics and Metabolism is one of the largest in the United States. This multidisciplinary team includes BMT, genetics, neurology, ophthalmology, neuropsychology, orthopedics, nutrition, physical therapy, social work, and ethics program members.

Graft-Versus-Host Disease

David Jacobsohn, MD, ScM , Chair, GVHD Committee, Pediatric Blood and Marrow Transplant Consortium

Kirsten Williams, MD

The BMT program has significant expertise in graft-versus-host disease. Dr. Jacobsohn has led national trials studying novel treatments of refractory graft-versus-host disease in children. Furthermore, he has published extensively on risk factors studying outcomes in GVHD in children. Dr. Williams is leading national trials on the treatment for lung GVHD. These studies translate into the ability to improve outcomes in GVHD. Children's National is one of the first institutions in the country to have a dedicated extracorporeal photopheresis program (ECP), offering ECP with the new CellEx machine to even very low-weight children. ECP is one of the first modalities used for treatment of steroid-refractory GVHD, which has been found to be well-tolerated and lead to fewer infections than other types of systemic immunosuppression.

The Shirley and William Howard Hematopoietic Stem Cell Laboratory

Brett Loechelt, MD, Director, Shirley and William Howard Cellular Therapy Laboratory

Kathy Mintz, MT, (ASCP), SBB, Manager

The Hematopoietic Stem Cell Laboratory consists of two clean room facilities, stem cell processing (ISO 8) and one for investigational new drug (IND) cellular therapies (ISO 7). The laboratory is a FACT (Foundation for the Accreditation of Cellular Therapy) accredited combined clinical and research facility.

The laboratory provides state-of-the-art cell processing of bone marrow, peripheral blood, umbilical cord blood, and donor lymphocyte for both autologous and allogeneic blood and marrow transplantations. As an advanced research facility, the laboratory has access to cutting-edge technology, including:

- CliniMACS® instrument, which provide the lab's capacity for sophisticated and rapid cellular product manipulation.
- Flow Cytometry Core, which includes a fluorescence-activated cell-sorting instrumentation that supplies rapid blood stem cell counts and analysis of stem cell components. In addition, the Hematopoietic Stem Cell Laboratory is accredited by Clinical Laboratory Improvement Amendments (CLIA) and the College of American Pathologists (CAP).

The Good Manufacturing Practice Facility for Immunotherapy

Patrick Hanley, PhD, Director, CETI Good Manufacturing Practice (GMP) Facility

The GMP Facility for Immunotherapy is a facility geared towards treating patients using IND-based cellular therapies. These therapies include T cells that target and treat viral infections and virus-related malignancies as well as T cells that target leukemia. The facility also is developing Mesenchymal Stromal Cells (MSCs), which are immunosuppressive cells that are being tested as a treatment for graft-versus-host-disease after bone marrow transplant, inflammatory bowel disease, and other regenerative medicine applications.

Clinical Team

David Jacobsohn, MD, ScM, Division Chief

Allistair Abraham, MD

Kristen Barbieri, PA-C

Catherine M. Bollard, MD

Johanna Curtis, NP-C

Jacqueline Dioguardi, PA-C

Brett J. Loechelt, MD

Evelio D. Perez-Albuerne, MD, PhD

Cheryl Reggio, RN

Amanda Sevinsky, RD, LD, CNSC

Regina Tosca, LGSW

Kirsten Williams, MD

Bonnie Yates, CPNP-AC

All patients can also be referred by contacting our transplant coordinator:

Samrawit Berhane

202-476-5456

sberhane@ChildrensNational.org

Program for Cell Enhancement and Technologies for Immunotherapy (CETI)

Catherine M. Bollard, MBChB, MD, FRACP, FRCPA, Director

The Program for Cell Enhancement and Technologies for Immunotherapy (CETI) seeks to understand how to co-opt the body's own immune system to combat cancer, infections, and disorders of immunity for example graft-versus host disease (GvHD), autoimmunity and immune deficiency. The Center also focuses on developing cell therapy for regenerative medicine covering ischemic disorders, tissue damage, and wound healing. The goal is to facilitate translational research, moving novel cell therapies from the bench to the clinic for a broad range of diseases from cancer and post transplant complications to chronic inflammatory and infectious diseases and new applications of cell therapy for cardiac, neurological, and pulmonary diseases.

Bone Marrow Harvests

In bone marrow harvests, stem cells are harvested directly from the red marrow in the crest of the ileum under general anesthesia. The procedure is minimally invasive and does not require stitches afterwards. Depending on the donor health and reaction to the procedure, the actual harvest can be an outpatient procedure or may require an overnight stay in the hospital to recover. Bone marrow harvests are most commonly performed on a relative who is the bone marrow donor for a patient needing an allogeneic transplant, but they also are performed on selected patients who will receive an autologous transplant.

Peripheral Blood Stem Cell Collection

Blood-forming stem cells also can be collected from the peripheral blood of a patient or donor using a procedure called apheresis. This is the usual way to collect stem cells for later autologous transplant, and it can be used for selected donors for allogeneic transplants as well. A multidisciplinary team, including specialists from BMT, Transfusion Medicine, Interventional Radiology, and Critical Care Medicine, works together to perform peripheral blood stem cell (PBSC) collection procedures. More than 150 PBSC collections have been performed by the center in the last six years. Children's has developed the ability to perform autologous PBSC collection safely even from small infants. The laboratory at Children's processes peripheral blood stem cells for blood and marrow transplantations.

Consultations and Referrals for Transplantation

Leukemia/Lymphoma

Dr. David Jacobsohn

202-476-6250

dajacobs@ChildrensNational.org

Dr. Kirsten Williams

202-476-4952

kmwillia@ChildrensNational.org

Solid Tumors

Dr. Evelio Perez-Albuerne

202-476-5887

eperez@ChildrensNational.org

Sickle Cell Disease

Dr. Allistair Abraham

202-476-5772

aabraham@ChildrensNational.org

Immunodeficiency and Inborn Errors of Metabolism

Dr. Brett Loechelt

202-476-2805

bloechel@ChildrensNational.org

Cellular Therapy

Dr. Catherine Bollard

202-476-4776

cbollard@ChildrensNational.org