



# The Blood and Marrow Transplant Program

Improving patient outcomes by transforming transplant care



*When your child needs a hospital, everything matters.<sup>SM</sup>*



### JOVAN CARPENTER

*Age 1, from Columbus, OH*

*The genetics team at Nationwide Children's Hospital diagnosed Jovan with Hurler Syndrome, a metabolic disorder causing deficient enzyme production that affects growth and development. Jovan initially received enzyme replacement therapy followed by a matched unrelated (MUD) umbilical cord blood (UCB) transplant at 9 months of age. He is now one year from allogeneic transplant and has made a full recovery with normal enzyme levels.*





## BMT AT NATIONWIDE CHILDREN'S HOSPITAL

The Blood and Marrow Transplant Program is at the core of the Division of Hematology, Oncology and BMT. From central nervous system malignancies and leukemia to nonmalignant hematologic disorders and inherited metabolic disorders, BMT at Nationwide Children's is intricately involved in bringing best outcomes to children requiring hematopoietic cell transplants.

The goal of the BMT Program is to provide comprehensive, compassionate care while advancing the field of hematopoietic cell transplantation through research that makes a difference in a patient's life.

The BMT Program at Nationwide Children's includes a 12-bed inpatient unit and outpatient clinic dedicated to BMT patients. This unit is located in the world-class, state-of-the-art hospital, which opened in 2012. With the support of the whole hospital, including the pediatric intensive care unit (PICU) and surgical services, we have the resources to treat the patient no matter what need arises.

**BMT can be associated with significant, life-threatening side effects. The care provided by our BMT team is critical to how well the patient will respond to therapy. Here, we work diligently through patient care, quality improvement and evidence-based research to alleviate or prevent detrimental side effects, not only providing life-saving treatment but also focusing on quality of life during and after transplant.**

*Jeffery J. Auletta, MD, Director, Blood and Marrow Transplant Program*

### MISSION STATEMENT

The BMT Program is committed to utilizing hematopoietic cell transplantation as the definitive cure for pediatric and young adult malignant and non-malignant disorders, as well as optimizing its clinical application by reducing transplant-associated morbidity and mortality through novel therapies and approaches in patient care.



## TRANSLATIONAL RESEARCH

The BMT Program is built upon three translational research pillars: supportive care, the Host Defense Program and the Cellular Therapy Program. By integrating care, research and collaboration at all levels, we are able to elevate the patient's opportunities for best outcomes and advance the field of BMT.

### *Supportive Care*

A team of researchers, physicians, nurses and support staff focus on treating and preventing infection, late effects and organ toxicity. As a site for pharmaceutical trials and protocol development in each of these areas, the team ensures that patients have every opportunity for the best possible outcomes.

Among the advanced therapies offered is extracorporeal photopheresis, a form of apheresis and photodynamic therapy in which the blood is treated with a photosensitizing agent and irradiated before it is returned to the body. Recent studies show that photopheresis is a promising treatment for graft versus host disease (GvHD). Under the leadership of Hemalatha Rangarajan, MD, photopheresis is being used to treat both GvHD and autoimmune skin disorders.

### *Host Defense Program*

The Host Defense Program (HDP) is a translational research collaborative among the Divisions of Hematology, Oncology and BMT and Infectious Diseases, The Research Institute at Nationwide Children's and The Ohio State University Comprehensive Cancer Center–Arthur G. James Cancer Hospital and Richard J. Solove Research Institute (OSUCC-James). The goal of the program is to reduce infection-related morbidity and mortality in immunocompromised patients through the use of novel therapeutics.

Areas of translational research focus in the HDP include:

- Immune modulation following hematopoietic cell transplants
- Gastrointestinal immunity and influence of the microbiome
- Viral pathogenesis

### *Cellular Therapy*

The newly created Cellular Therapy Program is led by Dean A. Lee, MD, PhD. This collaborative effort with OSUCCC-James is dedicated to pursuing clinical trials and innovative treatments.

Advanced cellular therapy uses knowledge of the immune system and the transplant process to target specific cells to prevent or reduce GvHD, infection and disease relapse. The implications of cellular therapy are broad and further development and refinement in this field will directly impact children everywhere.

Through my new position at Nationwide Children's, I am excited to bring together my clinical and research interests to develop trials of adoptive immune therapy and other cellular derived therapies for children undergoing hematopoietic stem cell transplantation.

Dean A. Lee, MD, PhD, Director, Cellular Therapy Program

## THE CARE TEAM: TREATMENT BEYOND DIAGNOSIS

Behind every patient is a compassionate team of doctors, nurses, researchers, therapists, social workers and support staff, all focused on providing individualized care and support. In addition to expert medical care, specialized support through Child Life and Nursing Touch is provided to all patients. Psychosocial support, including talk, art, music and massage therapies, is an integrated part of each patient's care. Specialized laboratory services support the clinical and research teams by enabling in-house access to testing and diagnostics.

From school education, family and sibling support groups, to help navigating health care and insurance and advanced illness management, the BMT Program at Nationwide Children's is truly holistic.

**A positive support system is essential for a positive outcome. We do everything we can to support the patient and his or her family throughout the continuum of complex transplant care. We partner with families that entrust us with the care of their children.**

*Dr. Auletta*

## QUALITY IMPROVEMENT: DEVELOPING THE CANCER CARE AND BMT INDEXES

The Division of Hematology, Oncology and BMT has created a patient- and family-centered strategic plan to track adherence to a broad range of best-practice guidelines, quality indicators and safety initiatives, called the Cancer Care Index (CCI). The CCI is designed as a meaningful measure of the program's impact on the oncology patient across the entire spectrum of care, from the time of diagnosis to the end of treatment, including death. The CCI aggregates patient harm events and the number of omissions of optimal care into a composite score that reflects overall program performance without regard to cancer type or patient outcome.

Fifteen domains were selected in three areas:

- Treatment-related quality and safety
- Providing a harm free environment
- Psychosocial support

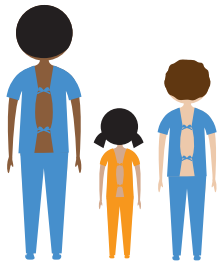
Through quality improvement projects in each of the domains involving all levels of staff and patient care, a two-year 42 percent reduction in CCI was obtained (2012-2014). This success demonstrates the ability to avoid preventable harm while consistently providing evidence-based care and psychosocial support. At Nationwide Children's, CCI has improved staff awareness about many sometimes overlooked cancer care domains. Using the CCI as an outcome metric in combination with robust quality improvement efforts has improved the overall performance of our Oncology and BMT Programs.

Like the CCI, the BMT Index includes 12 specific quality improvement projects that follow the care plan of the patient, from pre- to post-transplant. Quality metric areas include fertility consultations, adherence to standardized treatment regimens, infection prevention and revaccination.



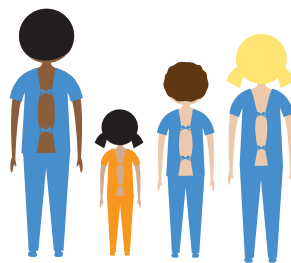
# The Blood and Marrow Transplant Program

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**98.6%**  
**Survival**

at 100 days post-transplant  
for the last 5 years across all  
transplant types



**100%**  
**Survival**

at 100 days post-transplant for  
the last 5 years for malignant  
conditions



**6**  
**Credentialed  
Transplant  
Physicians**

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**10** Consortia &  
Investigator Initiated  
**Clinical Trials**

**12** BMT-specific  
**Quality  
Improvement**  
Projects

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**235** **Total  
Transplants**  
2010-2015

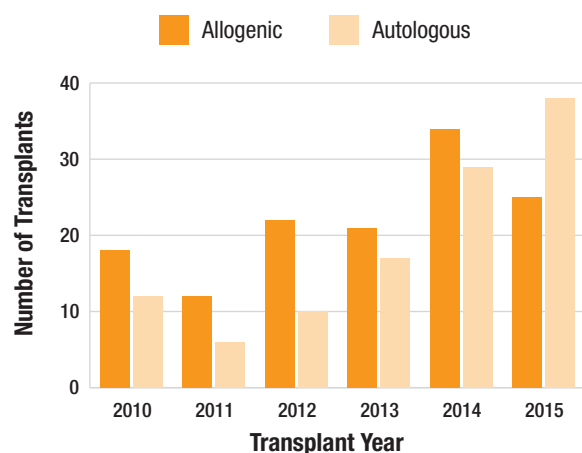


## BMT at Nationwide Children's: At a Glance

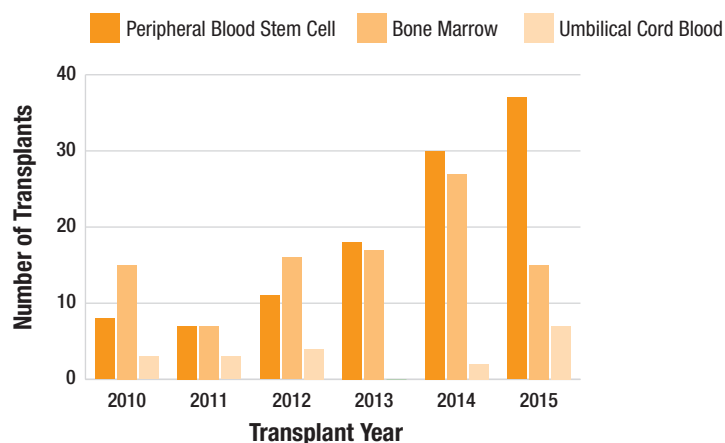
Established in 1992, the BMT Program at Nationwide Children's has a history of excellence and dedication to best outcomes.

\*All chart information based on 2015 data.

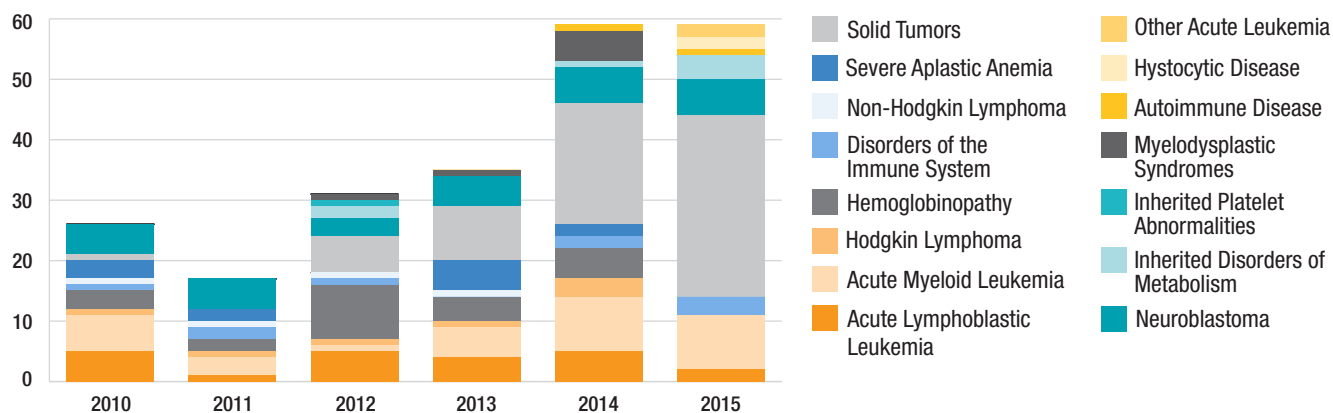
### Autologous & Allogeneic Transplants



### Stem Cell Grafts Infused



### Transplant Diagnoses



### Transplantation Outcomes (percent)

	2011	2012	2013	2014
<b>Autologous – Malignant</b>				
100 Day Overall Survival	100	100	100	100
1 Year Overall Survival	80	100	100	88
<b>Allogeneic – Malignant</b>				
100 Day Overall Survival	100	100	100	100
1 Year Overall Survival	92	75	70	86
<b>Allogeneic – Nonmalignant</b>				
100 Day Overall Survival	100	92	100	100
1 Year Overall Survival	86	83	100	94
<b>Total</b>				
100 Day Overall Survival	100	93	100	100
1 Year Overall Survival	88	87	91	89

## WELL-CONNECTED

As the pediatric affiliate of the NCI-designated OSUCCC-James, the BMT Program at Nationwide Children's is connected to one of the premier adult cancer institutions in the country, thus enabling an unrivaled continuum of care.

Additionally, the BMT Program is part of numerous national and international consortia. This collaboration and reach enables the team to provide the most current therapies and opportunities for clinical trial participation. With approximately 74 percent of Nationwide Children's eligible oncology patients enrolled in a clinical trial, Nationwide Children's Division of Hematology, Oncology and BMT is among the leading cancer centers for clinical trial enrollment by the Children's Oncology Group.

### *National and International Affiliations:*



The world's childhood cancer experts



BE  THE MATCH®



Primary Immune Deficiency Treatment Consortium



pediatric blood & marrow transplant consortium



Active participation and leadership of BMT program faculty and staff at the local, national and international level:

- Operations Committee, Center for Regenerative Medicine and Cell-based Therapies, The Ohio State University
- Social Work Education Committee and Patient Education Advisory Group, National Marrow Donor Program (NMDP)
- American Cancer Society Central Ohio Patient Services Committee and the Central Ohio Transportation Committee
- Data Audit Committee, Foundation for the Accreditation of Cellular Therapy (FACT)-Center for International Blood and Marrow Transplant Research (CIBMTR)
- National Advisory Steering Committee, Improving Pediatric Sepsis Outcomes, Children's Hospital Association (CHA)
- Medical Advisory Board, The Bone Marrow Foundation (BMF)
- Steering Committee, Pediatric Blood and Marrow Transplant Consortium (PBMTTC)
- Supportive Care Group, PBMTTC
- Cellular Therapy Group, PBMTTC
- Infection and Immune Reconstitution Working Committee (INWC), Center for International Blood and Marrow Transplant Research (CIBMTR)
- Executive Committee, Pediatric Acute Lung Injury and Sepsis Initiative (PALISI)
- Cellular Therapies Committee, Westhafen Intercontinental Group
- NIH Recombinant DNA Advisory Committee





# Bone Marrow Transplant Clinic

## The Faces of BMT at Nationwide Children's

Treating the diverse needs of our patients is at the heart of what we do. From weeks-old infants with metabolic syndromes to teenagers fighting AML, we have the opportunity, resources and drive to offer treatments that save lives.

*Rolla F. Abu-Arja, MD, Clinical Director, Blood and Marrow Transplant Program*





**GRIFFIN HAYDEN**

*Age 16, from Newark, OH*

*Griffin was diagnosed with relapsed, refractory AML. He received two transplants at Nationwide Children's Hospital. He attained full remission after his second transplant, and he is now 18 months out and doing well.*





### IVY MOSS

*Age 8, from Toledo, OH*

*Ivy was diagnosed with refractory T-cell acute lymphoblastic leukemia (ALL). Ivy is from a mixed race and had a difficult bone marrow donor search. She underwent a mismatched unrelated donor (MMUD) umbilical cord blood transplant. Ivy is over one year from transplant and has minimal complications.*





**ALEX CARMAN**

*Age 12, from Toledo, OH*

*Alex was diagnosed with high-risk AML and had multiple relapses. He underwent a matched unrelated (MUD) bone marrow transplant followed by donor lymphocyte infusions (DLI) that ultimately resulted in a long-term disease remission. Alex is now four years from this therapy, and he just finished 6th grade. He continues to require medical management for his chronic graft versus host disease and in the long-term follow up clinic.*





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## Referrals and Consultations

Online: [NationwideChildrens.org/Hematology-Oncology-BMT](https://www.NationwideChildrens.org/Hematology-Oncology-BMT)

Phone: (614) 355-1272 | Fax: (614) 722-3369

Physician Direct Connect Line for 24-hour urgent physician consultations:  
(614) 355-0221 or (877) 355-0221.

