

# Morgan Stanley Children's Hospital at NewYork-Presbyterian

## Focus on Pediatrics

Affiliated with Columbia University College of Physicians and Surgeons

*Pediatric Blood and Marrow Transplantation Issue*

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## New Advances in Reduced Intensity Conditioning Prior to Allogeneic Stem Cell Transplantation in Children

Allogeneic hematopoietic stem cell transplantation has curative potential for various malignant and non-malignant diseases. Hematopoietic stem cells can be obtained from cord blood, bone marrow or stem cells mobilized in the peripheral blood circulation.

In the early 1970's patients received very high doses of chemotherapy as conditioning prior to bone marrow transplant. At that time the thought was that high doses of chemotherapy were required to get rid of leukemia. However, high dose chemotherapy itself was associated with significant morbidity and high mortality. Over the last 2 decades we have learned that donor [ continued on page 3 ]

### A Message from the Chief

Dear Colleagues and Friends,

Welcome to the inaugural edition of the Division of Blood and Marrow Transplantation at the Morgan Stanley Children's Hospital at NewYork-Presbyterian/Columbia University Medical Center newsletter. On behalf of the almost 80 devoted staff members in our program we would like to introduce you to some of the features and highlights of our teams' efforts and successes.

We have a very active program in basic, translational and clinical research in hematology, oncology, immunology, stem cell biology and transplantation and provide state-of-the-art quality care in these clinical areas. Our multidisciplinary team includes Physicians, Physician Scientists, Pediatric Hematology/Oncology fellows in training, Nurses, Advanced Practice, Quality Assurance, and Clinical Research Nurses and Associates, Social Workers, Psychologists, Clinical Pharmacists, Nutritionists, Basic and Translational Scientists, and Biostatisticians.

Our areas of special interest include stem cell transplantation for malignant and non-malignant disease, reduced intensity conditioning, targeted immunotherapy, adoptive cellular immunotherapy, stem cell regenerative therapy, supportive care, graft vs. host and graft vs. tumor biology, tumor immunogenetics, developmental therapeutics and molecular biology of childhood leukemias and lymphomas. We are a FACT, NMDP and COG accredited program. Each year we publish 10-20 manuscripts and have 30-40 national and international peer review presentations.

In this issue we feature our unique investigation of utilizing reduced intensity conditioning instead of fully myeloablative conditioning in preparation for stem cell/bone marrow transplant and the wonderful success story of one of our patients. Please feel free to contact me if you wish to receive any other additional information about our program.

Best Regards,

Mitchell S. Cairo, MD

Chief, Division of Pediatric Blood and Marrow Transplantation

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## Patient Profile

# Third Time was a Charm: Brotherly Love

For the Pena family, monthly visits to the hematologist and frequent hospitalizations were a way of life. Two of their three sons live with sickle cell disease (Hgb SC) and G6PD deficiency. Charlie, now 15 years of age, was diagnosed with sickle cell disease before birth by amniocentesis and with G6PD at 1 year of age. Michael, now 12 years of age, was diagnosed with sickle cell disease at birth and 2 months later with G6PD.

Until 2 years ago these monthly visits and frequent hospitalizations for complications of their disease were the norm for Charlie and Michael. At that time Charlie began experiencing pain crises with increased severity and frequency. His increase in pain prompted his mother to consider bone marrow transplant for long-term treatment and potential cure of his disease. During the past 2 decades, bone marrow transplant has been increasingly used for the long-term treatment and cure of sickle cell disease. Bone marrow transplantation from a matched sibling is considered curative and effective for sickle cell disease. Most of the side effects seen with

*Charlie, Michael  
and Kyle*



bone marrow transplant are directly related to the high-intensity of chemotherapy used as conditioning to receive donor stem cells. Dr. Cairo and his team have been treating this group of patients with a lower dose chemotherapy regimen before transplant for the past 7 years with 80-90% success.

The search for a suitable donor yielded a perfect match from the third and youngest brother Kyle. Charlie had his transplant a little over a year ago and is doing quite well. Michael received his transplant a little over 2 months ago and is on his way to recovery. Kyle's gift of love has resulted in full engraftment of his donor cells for each of his brothers.

*Leah Violago MS, CPNP • Pediatric Blood and Marrow Transplant Nurse Practitioner at Morgan Stanley Children's Hospital*

## Philanthropy

**Pediatric Cancer Research Foundation (PCRF)** is a non-profit organization which was organized in 1982 and has raised over 22 million dollars for pediatric cancer research over the past 26 years. PCRF is a major supporter of the pediatric cancer research accomplished through our program. PCRF was involved in the initial development of and continue to support the Pediatric Cancer Research Foundation Laboratory at Columbia University of which Dr. Cairo is the Principal Investigator and Director. For more information about PCRF and how to get involved or contribute, please visit their web site at [www.pcrf-kids.com](http://www.pcrf-kids.com).

**Dreaming for Discovery and Cure (DDC)** is a group of parents, friends and members of the community who have developed a program to raise support for stem cell and cancer research. The DDC has many fundraisers throughout the year to raise awareness of the cutting edge research that is being completed everyday within our Blood and Marrow Transplant Research Program. For more information about DDC and how to get involved or contribute, please visit us at [www.dream-discover-cure.org](http://www.dream-discover-cure.org). The website includes a description of the DDC's goals in raising money for Blood and Marrow Transplant research. The website is also updated regularly with upcoming events and the latest news in Blood and Marrow Transplantation.



M.D. Profile  
**Mitchell S. Cairo, MD**

Mitchell S. Cairo, MD, is Professor of Pediatrics, Medicine, and Pathology at the Columbia University College of Physicians and Surgeons in New York, New York. He is Chief of the Division of Pediatric Blood and Marrow Transplantation at Morgan Stanley

Children's Hospital at New York-Presbyterian/Columbia University, as well as Director of the Pediatric Hematology/Oncology Fellowship Program. In addition, he is Medical Director of the National Marrow Donor Unrelated Transplant Program at Columbia.

Upon earning his medical degree from the School of Medicine at the University of California, San Francisco (UCSF), Dr. Cairo completed an internship and a residency in pediatrics at the University of California at Los Angeles Harbor General Hospital in Torrance, California, followed by a chief residency in the Department of Pediatrics at UCSF. He concluded his specialty



## I'll be your inspiration

Heather Abrams

### **New Advances** [continued from the cover]

T-lymphocytes and natural killer cells provide a graft-versus-malignancy effect and high doses of chemotherapy are not essential for the cure of various malignancies. When children receive very high dose chemotherapy followed by an allogeneic hematopoietic stem cell transplant, they suffer from numerous side effects including pain, fever, diarrhea, anorexia, infection, liver and kidney failure. In the last 7 to 8 years in our program we have used the approach of reduced intensity conditioning prior to allogeneic hematopoietic stem cell transplant for children with various malignant (acute and chronic myeloid leukemia, Hodgkin lymphoma, non-Hodgkin lymphoma and neuroblastoma) and non-malignant disorders (sickle cell anemia, thalassemia, aplastic anemia, congenital immunodeficiencies, metabolic and genetic disorders).

Our goal is to spare children from early morbidity and mortality and late morbidities. We were invited to present our experience in this field at a recent scientific meeting of the International Society of Pediatric Oncology in Berlin, Germany. We have learned that reduced intensity conditioning prior to allogeneic hematopoietic stem cell transplant is safe, is associated with less morbidity and more importantly, lower mortality, in the first 100 days after transplant (3%) with an overall outcome that is comparable to myeloablative allogeneic hematopoietic stem cell transplant. Due to its curative potential, reduced intensity conditioning might be helpful in children with non-malignant diseases such as sickle cell anemia, thalassemia and other genetic disorders.

*Written by Prakash Satwani, MD Assistant Professor of Pediatrics*

Lance Armstrong: the survivor of survivors. He beat a horrible bout of cancer and is still an amazing cyclist. But Lance Armstrong did have a certain chemotherapy drug: bleomycin. I did too. Bleomycin lowers your lung capacity.

Not only was I sick once, but I was sick twice with Hodgkin's disease. I had countless treatments with chemotherapy and radiation...and survived. I underwent 2 stem cell transplants. The first was with my own stem cells and with strong doses of chemotherapy. I was in the same hospital room for a month. But as promised, my stem cells started to grow 10 days later and there was no evidence of my disease. My second transplant was from an unrelated cord blood donor, but this time I had lower doses of chemotherapy so I didn't get as sick. But they did make my immune system very weak so I again had to be isolated to my hospital room for over a month. But at the end of my transplant journey...I was in remission and I have been in remission now for almost 8 years. Not only do I love what I do which is working with animals, but I am also a cycling/spinning instructor. I might not be able to do a Tour de France, but I can teach an hour long cycling class. And it's funny, I am participating in a 4 hour cycling marathon to benefit the Livestrong foundation. Sometimes it is hard to breathe, but I make it through. My favorite line to say to my class is, "If I can do it, you can do it!" My favorite song we cycle to is: I'll Be Your Inspiration. That is exactly what I try to be: inspirational.

Having cancer changes your life, and it definitely makes you appreciate things more than you ever did before. I am a stronger person for it. Life is a gift and I want to live it to its fullest.

training with an American Cancer Society fellowship in pediatric hematology/oncology at Indiana University in Indianapolis.

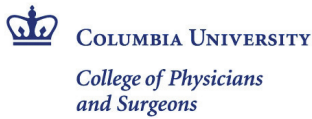
Dr. Cairo's clinical areas of expertise are pediatric immunology, childhood leukemia and lymphoma, especially non-Hodgkin lymphoma, blood and marrow transplantation, and developmental therapeutics. His research interests are focused on tumor and developmental immunology, autologous and allogeneic stem cell transplantation, and most recently umbilical cord blood transplantation. He is Chairman or Vice Chairman of several ongoing clinical trials affiliated with the Children's Oncology

Group. He is an active presenter, often invited to lecture nationally and internationally. Dr. Cairo is the author of more than 240 peer-reviewed articles and more than 700 abstracts.

Dr. Cairo is certified by both the American Board of Pediatrics and the subspecialty Board of Pediatric Hematology-Oncology. He is a member of more than a dozen professional national and international societies, including the American Academy of Pediatrics, American Society of Hematology, American Society of Clinical Oncology, just to name a few. He is a member of the Board of Directors of the International Society of Experimental Hematology.

# Honors

- **Monica Bhatia, MD**  
received the Physician of the Year award at the Morgan Stanley Children's Hospital at NewYork-Presbyterian in 2006.
- **Prakash Satwani, MD**  
Elected to the Editorial Board of the Journal of Bone Marrow Transplantation, Nature Publishing
- **Mitchell S. Cairo, MD**  
was elected to the Board of Directors of the International Society of Experimental Hematology in September 2008.



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on Pediatric Blood and  
Marrow Transplantation

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